

## IMPORTANT NOTICE

THIS OFFERING IS AVAILABLE ONLY TO INVESTORS WHO ARE (1) LOCATED OUTSIDE THE UNITED STATES AND EITHER (A) A SWISS RESIDENT OR (B) OUTSIDE SWITZERLAND PROVIDED SUCH AVAILABILITY IS PERMITTED UNDER APPLICABLE SECURITIES LAWS OR (2) "QUALIFIED INSTITUTIONAL BUYERS" (EACH A "QIB") (AS DEFINED IN RULE 144A ("RULE 144A") UNDER THE U.S. SECURITIES ACT OF 1933, AS AMENDED (THE "SECURITIES ACT"))).

**IMPORTANT:** You must read the following disclaimer before continuing. The following disclaimer applies to the attached offering memorandum (the "**Document**") following this page and you are therefore advised to read this disclaimer carefully before reading, accessing or making any other use of the Document. In accessing the Document, you agree to be bound by the following terms and conditions, including any modifications to them, any time you receive any information from us as a result of such access. You acknowledge that this electronic transmission and the delivery of the Document is confidential and intended only for you and you agree you will not reproduce, copy, download or publish this electronic transmission or the Document (electronically or otherwise) or forward or provide this electronic transmission or the Document to any other person.

NOTHING IN THIS ELECTRONIC TRANSMISSION CONSTITUTES AN OFFER OF, OR THE SOLICITATION OF AN OFFER TO BUY OR SUBSCRIBE FOR, SECURITIES TO ANY PERSON IN THE UNITED STATES, OR IN ANY OTHER JURISDICTION WHERE IT IS UNLAWFUL TO DO SO. THE SECURITIES DESCRIBED HEREIN HAVE NOT BEEN, AND WILL NOT BE, REGISTERED UNDER THE SECURITIES ACT, OR THE SECURITIES LAWS OF ANY STATE OR OTHER JURISDICTION OF THE UNITED STATES, AND SUCH SECURITIES MAY NOT BE OFFERED, SOLD, HYPOTHECATED OR OTHERWISE TRANSFERRED EXCEPT (1) PURSUANT TO AN EFFECTIVE REGISTRATION STATEMENT UNDER THE SECURITIES ACT, (2) IN ACCORDANCE WITH RULE 144A TO A PERSON THAT THE HOLDER AND ANY PERSON ACTING ON ITS BEHALF REASONABLY BELIEVES IS A QIB WITHIN THE MEANING OF RULE 144A THAT (A) WAS NOT FORMED FOR THE PURPOSE OF INVESTING IN THE SECURITIES AND (B) IS ACQUIRING THE SECURITIES FOR ITS OWN ACCOUNT OR FOR THE ACCOUNT OF A QIB, (3) IN AN OFFSHORE TRANSACTION IN ACCORDANCE WITH RULE 903 OR RULE 904 OF REGULATION S ("**REGULATION S**") UNDER THE SECURITIES ACT OR (4) IN ACCORDANCE WITH RULE 144 UNDER THE SECURITIES ACT, IF AVAILABLE, IN EACH CASE IN ACCORDANCE WITH ANY APPLICABLE SECURITIES LAWS OF ANY STATE OR TERRITORY OF THE UNITED STATES AND OF ANY OTHER JURISDICTION. THERE WILL BE NO PUBLIC OFFER OF THE SECURITIES IN THE UNITED STATES.

You have been sent the attached Document on the basis that you have confirmed to UBS AG (the "**Global Coordinator and Bookrunner**") and Mirabaud Securities Limited (the "**Co-Manager**" and, together with the Global Coordinator and Bookrunner, the "**Managers**" and each, a "**Manager**"), that (i) either (a) you are located outside the United States and you are (1) a Swiss resident or (2) a person outside Switzerland into whose possession the Document may be lawfully delivered in accordance with the laws of the jurisdiction in which you are located or (b) you are a "qualified institutional buyer" (as defined in Rule 144A) and (ii) you consent to delivery by electronic transmission.

You are reminded that the Document has been delivered to you on the basis that you are a person into whose possession the Document may be lawfully delivered in accordance with the laws of the jurisdiction in which you are located and you may not, nor are you authorized to, deliver the Document to any other person.

If you receive the Document by e-mail, you should not reply by e-mail to this announcement, and you may not purchase any rights or securities by doing so. Any reply e-mail communications, including those you generate by using the "reply" function on your e-mail software, will be ignored or rejected.

The attached Document is being provided to you on a confidential basis for informational use solely in connection with your consideration of the purchase of the securities referred to therein. Its use for any other purpose is not authorized, and you may not, nor are you authorized to, copy or reproduce the Document in whole or in part in any manner whatsoever or deliver, distribute or forward the Document or disclose any of its contents to any other person. Failure to comply with

this directive may result in a violation of the Securities Act or the applicable laws of other jurisdictions. If you are not the intended recipient of the Document, you are hereby notified that any dissemination, distribution or copying of the Document is strictly prohibited.

The Document does not constitute or contain any offer to sell or invitation to subscribe or make commitments for or in respect of any security in any jurisdiction where such an offer or invitation would be unlawful. If a jurisdiction requires that the offering be made by a licensed broker or dealer and the Managers or any affiliate of the Managers is a licensed broker or dealer in that jurisdiction, the offering shall be deemed to be made by the Managers or such affiliate on behalf of Santhera Pharmaceuticals Holding AG, a stock corporation (*Aktiengesellschaft*) organized under the laws of Switzerland in accordance with articles 620 et seq. Swiss Code of Obligations with its seat in Pratteln, Canton of Basel-Landschaft (the "**Company**") in such jurisdiction.

The attached Document has been sent to you in an electronic form. You are reminded that documents transmitted via this medium may be altered or changed during the process of electronic transmission and, consequently, neither the Company nor the Managers, nor any person who controls any of them nor any director, officer, employee nor agent of any of them, nor any affiliate of any such person accepts any liability or responsibility whatsoever in respect of any difference between the Document distributed to you in electronic format and the hard copy version.

Restriction: Nothing in this electronic transmission constitutes, and may not be used in connection with, an offer of securities for sale to persons other than the specified categories of recipients described above and to whom it is directed and access has been limited so that it shall not constitute a general solicitation. If you have gained access to this transmission contrary to the foregoing restrictions, you will be unable to purchase any of the securities described therein.

No representation or warranty, express or implied, is made by the Managers or any of their respective affiliates or advisers as to the accuracy or completeness of this information, and nothing contained in the Document is, or may be relied upon as, a promise or representation in this respect, whether as to the past or the future, by the Managers or by their respective affiliates or advisers. The Managers assume no responsibility for the Document's accuracy, completeness or verification and accordingly disclaim, to the fullest extent permitted by applicable law, any and all liability whether arising in tort, contract or otherwise which they might otherwise be found to have in respect of the Document or any such statement.

The Managers are acting exclusively for the Company and no one else in connection with the offering. They will not regard any other person (whether or not a recipient of the Document) as their client in relation to the offering and will not be responsible to anyone other than the Company for providing the protections afforded to its clients nor for giving advice in relation to the offering or any transaction or arrangement referred to herein.

You are responsible for protecting against viruses and other destructive items. Your receipt of the Document via electronic transmission is at your own risk and it is your responsibility to take precautions to ensure that it is free from viruses and other items of a destructive nature.

**Santhera Pharmaceuticals Holding AG**  
(a stock corporation organized under Swiss law)



**Listing of 1,000,000 registered shares with a nominal value of CHF 1.00 each**  
**Offering and listing of up to 5,000,000 registered shares with a nominal value of CHF 1.00 each**

This offering and listing memorandum (the “**Offering Memorandum**”), which has been prepared in accordance with the listing rules (the “**Listing Rules**”) of the SIX Swiss Exchange Ltd (the “**SIX Swiss Exchange**”) and their implementing provisions and the Swiss Code of Obligations of March 30, 1911, as amended (the “**CO**”) relates to (i) the listing (the “**Listing**”) of (A) 1,000,000 new registered shares of Santhera Pharmaceuticals Holding AG (the “**Company**” and, together with the Company’s subsidiaries, the “**Group**”, “**Santhera**”, “**we**” or “**us**”) with a nominal value of CHF 1.00 each (the “**Idorsia Shares**”, any such registered share of the Company a “**Share**”, ISIN CH0027148649, Swiss Security Number 2714864, ticker symbol SANN) on the SIX Swiss Exchange according to its International Reporting Standard (the “**International Reporting Standard**”) and (B) of up to 5,000,000 new registered shares of the Company with a nominal value of CHF 1.00 each (the “**Offered Shares**” and, together with the Idorsia Shares, the “**New Shares**”) and (ii) the offering (the “**Offering**”) of the Offered Shares. The 1,000,000 Idorsia Shares were issued in a private placement to Idorsia Pharmaceuticals Ltd (“**Idorsia**”) on November 21, 2018 as equity consideration for the acquisition from Idorsia of an option for the exclusive sub-license relating to ReveraGen BioPharma, Inc.’s (“**ReveraGen**”) dissociative steroid vamorolone, as further described in this Offering Memorandum. The Idorsia Shares are not being offered pursuant to this Offering Memorandum. The Offered Shares have been resolved to be created at an extraordinary shareholders’ meeting of the Company held on December 11, 2018.

The Company targets gross proceeds from the Offering of approximately CHF 50 million (based on the maximum number of Offered Shares and the closing price of the Shares on the SIX Swiss Exchange on December 11, 2018). The Company intends to use the net proceeds of the sale of the Offered Shares to fund the USD 20.0 million cash component of the consideration to Idorsia for the acquisition of the option for the exclusive sub-license to ReveraGen’s vamorolone and any net proceeds from the Offering in excess of that amount for general corporate purposes, as further described in this Offering Memorandum. The Company intends to complete the Offering as long as the net proceeds exceed the CHF equivalent of USD 20 million. If the Company is unable to raise the targeted proceeds in this Offering, the Company will be required to raise additional funds (equity and/or debt financing) in the immediate short term in order to continue its operations as planned. See “*Risk Factors—Risks related to our business and operations—Risks related to our financial position, capital needs and transactions*” beginning on page 11. The New Shares and all existing Shares are fungible and rank *pari passu* in all respects with each other.

The Offering consists of (i) a public offering in Switzerland, (ii) private placements in certain jurisdictions outside the United States of America (the “**United States**” or the “**U.S.**”) and Switzerland in accordance with applicable securities laws and in reliance on Regulation S (“**Regulation S**”) under the U.S. Securities Act of 1933, as amended (the “**Securities Act**”), and on the basis of exemptions provided by directive 2003/71/EC of the European Parliament and the Council of November 4, 2003 on the prospectus to be published when securities are offered to the public or admitted to trading, as amended (the “**Prospectus Directive**”), and (iii) private placements within the United States to qualified institutional buyers (“**QIBs**”) as defined in, and in reliance upon, the exemption from the registration requirements of the Securities Act provided by Rule 144A under the Securities Act (“**Rule 144A**”).

The offer price per Offered Share (the “**Offer Price**”) for the Offered Shares will be determined by the Company together with UBS AG (the “**Global Coordinator and Bookrunner**”) and Mirabaud Securities Limited (the “**Co-Manager**” and, together with the Global Coordinator and Bookrunner, the “**Managers**” and each, a “**Manager**”) following an accelerated bookbuilding process. The Company expects to publish the Offer Price and the final number of Offered Shares sold in the Offering by electronic media, by press release and in a pricing supplement to this Offering Memorandum on or around December 14, 2018. This Offering Memorandum and the pricing supplement thereto will constitute the final offering and listing memorandum.

Application has been made to, and approval has been given subject to certain conditions by, the SIX Swiss Exchange to list the New Shares on the SIX Swiss Exchange according to the International Reporting Standard. The Idorsia Shares were listed, and trading in the Idorsia Shares commenced, on the SIX Swiss Exchange on November 22, 2018. The Company expects that the Offered Shares will be listed, and trading in the Offered Shares will commence, on the SIX Swiss Exchange on or around December 18, 2018 (the “**First Day of Trading**”). All New Shares will be traded together with the existing Shares on the SIX Swiss Exchange in Swiss francs and settle and clear through SIX SIS Ltd (“**SIS**”).

The Offered Shares will be issued as uncertificated securities (*Wertrechte*) within the meaning of article 973c of the Swiss Code of Obligations (the “**CO**”) and established as intermediated securities (*Bucheffekten*) within the meaning of the Swiss Federal Intermediated Securities Act of October 3, 2008, as amended (the “**FISA**”; *Bucheffektengesetz*). It is expected that delivery of the Offered Shares in the form of intermediated securities against payment of the Offer Price will be made in book-entry form through the facilities of SIS on or around December 18, 2018 (the “**Closing Date**”).

**Investing in the Shares involves risks. For a discussion of certain factors that should be considered in deciding whether to invest in the Shares, see “Risk Factors” beginning on page 11.** For a description of certain restrictions regarding the offering and sale of the Offered Shares and the resale and transfer of the Shares, see “*Notice to Prospective Investors*” and “*Selling and Transfer Restrictions*” beginning on pages iv and 151, respectively.

**The Shares have not been and will not be registered under the Securities Act, or with any securities regulatory authority of any state or other jurisdiction in the United States, and are being offered and sold in the United States only to QIBs in reliance on Rule 144A and outside the United States in reliance on Regulation S. Prospective purchasers that are QIBs are hereby notified that the seller of the Shares may be relying on the exemption from the provisions of Section 5 of the Securities Act provided by Rule 144A. For additional information regarding selling and transfer restrictions, see “*Selling and Transfer Restrictions*” beginning on page 151.**

---

*Global Coordinator and Bookrunner*

**UBS AG**

*Co-Manager*

**Mirabaud Securities Limited**

---

Offering and Listing Memorandum dated December 12, 2018

<b>Important Information about this Offering Memorandum.....</b>	<b>i</b>
<b>Availability of Documents.....</b>	<b>iii</b>
<b>Notice to Prospective Investors.....</b>	<b>iv</b>
<b>Service of Process and Enforcement of Civil Liabilities under U.S. Law.....</b>	<b>ix</b>
<b>Third-Party Data.....</b>	<b>x</b>
<b>Forward-Looking Statements.....</b>	<b>x</b>
<b>Presentation of Financial and Other Information.....</b>	<b>xi</b>
<b>Exchange Rate Information.....</b>	<b>xii</b>
<b>Share Price Information.....</b>	<b>xiii</b>
<b>I. Summary.....</b>	<b>1</b>
<b>II. Summary of the terms of the Offering.....</b>	<b>4</b>
<b>III. Summary of Financial Information.....</b>	<b>8</b>
<b>IV. Risk Factors.....</b>	<b>11</b>
<b>V. Use of Proceeds.....</b>	<b>37</b>
<b>VI. Dividends and Other Distributions.....</b>	<b>38</b>
<b>VII. Capitalization and Indebtedness.....</b>	<b>39</b>
<b>VIII. Selected Financial Data.....</b>	<b>40</b>
<b>IX. Management’s Discussion and Analysis of Financial Condition and Results of Operations.....</b>	<b>43</b>
<b>X. The Company and its Business.....</b>	<b>65</b>
<b>XI. Legal and Regulatory Environment.....</b>	<b>97</b>
<b>XII. The Issuance of the New Shares.....</b>	<b>117</b>
<b>XIII. Related Party Transactions.....</b>	<b>118</b>
<b>XIV. Principal Shareholders.....</b>	<b>119</b>
<b>XV. Board of Directors and Executive Management.....</b>	<b>122</b>
<b>XVI. Description of the Company’s Capital Structure and Shares.....</b>	<b>133</b>
<b>XVII. SIX Swiss Exchange.....</b>	<b>145</b>
<b>XVIII. Offering and Sale.....</b>	<b>147</b>
<b>XIX. Selling and Transfer Restrictions.....</b>	<b>151</b>
<b>XX. Taxation.....</b>	<b>156</b>
<b>XXI. General Information.....</b>	<b>164</b>
<b>Index to Financial Statements.....</b>	<b>F-1</b>

## IMPORTANT INFORMATION ABOUT THIS OFFERING MEMORANDUM

The Company assumes responsibility for the completeness and accuracy of this Offering Memorandum pursuant to article 27 of the Listing Rules and section 4 of Scheme A thereunder. The Company confirms that, to the best of its knowledge, the information contained in this Offering Memorandum is correct and that no material facts or circumstances have been omitted therefrom.

This Offering Memorandum has been prepared in accordance with the Listing Rules and their implementing provisions to the extent applicable and the CO for the purposes of offering the Offered Shares and listing the Offered Shares on the SIX Swiss Exchange according to the International Reporting Standard.

The Offering consists of (i) a public offering in Switzerland, (ii) private placements in certain jurisdictions outside the United States and Switzerland in accordance with applicable securities laws and in reliance on Regulation S, and on the basis of exemptions provided by the Prospectus Directive, and (iii) private placements within the United States only to QIBs as defined in, and in reliance upon, the exemption from the registration requirements of the Securities Act provided by Rule 144A. Except in connection with the offering and sale of the Offered Shares in Switzerland, no action has been or will be taken in any jurisdiction by the Company or the Managers that would permit a public offering of the Offered Shares or possession or distribution of this Offering Memorandum or any other publicity materials relating to the Offering in any country or jurisdiction where action for such purpose is required. Persons in possession of this Offering Memorandum are required to inform themselves about, and to comply with, any applicable laws that restrict the distribution of this Offering Memorandum and the offer and sale of the Offered Shares. None of the Company, the Managers and their respective affiliates accepts any legal responsibility for any violation of such restrictions. For a description of the restrictions on resale and transfer of the Offered Shares, see “*Notice to Prospective Investors*” and “*Selling and Transfer Restrictions*” beginning on pages iv and 151, respectively. The Idorsia Shares have already been sold and are not being offered pursuant to this Offering Memorandum.

The information contained in this Offering Memorandum has been provided by the Company and by the other sources identified in this Offering Memorandum. No representation or warranty, express or implied, is made by the Managers or any of their respective affiliates or advisers as to the accuracy or completeness of this information, and nothing contained in this Offering Memorandum is, or may be relied upon as, a promise or representation in this respect, whether as to the past or the future, by the Managers or by their respective affiliates or advisers.

Each prospective investor in the Offered Shares outside Switzerland, by accepting delivery of this Offering Memorandum, will be deemed to have acknowledged, represented to and agreed with the Company and the Managers that:

- (i) this Offering Memorandum is personal to such prospective investor and does not constitute an offer to any other person, or to the public generally, to purchase or otherwise acquire the Offered Shares outside Switzerland. Distribution of this Offering Memorandum or disclosure of any of its contents to any person other than such prospective investor and those persons, if any, retained to advise such prospective investor with respect thereto is unauthorized, and any disclosure of any of its contents, without the prior written consent of the Managers is prohibited;
- (ii) the prospective investor shall not make any photocopies or electronic copies of this Offering Memorandum or any documents referred to herein (other than for its own use); and
- (iii) the prospective investor shall not forward or deliver this Offering Memorandum (in any form) to third parties.

The information contained in this Offering Memorandum is accurate only as of the date of this Offering Memorandum. Neither the delivery of this Offering Memorandum nor any sale made hereunder shall, under any circumstances, create any implication that there has been no change in the affairs of the Group since the date hereof or that the information contained herein is correct as of any time after the date hereof. Any notices containing or announcing amendments or changes to the terms of the Offering or to this Offering

Memorandum will be announced through electronic media. Notices required under the Listing Rules will be published on the website of the SIX Swiss Exchange (currently: [https://www.six-group.com/exchanges/news/official\\_notices/search\\_en.html](https://www.six-group.com/exchanges/news/official_notices/search_en.html)). Any such notice will constitute an integral part of this Offering Memorandum.

The Company expects to publish a pricing supplement to this Offering Memorandum on or around December 14, 2018. This Offering Memorandum and the pricing supplement will constitute the final offering and listing memorandum in relation to the Offered Shares.

## AVAILABILITY OF DOCUMENTS

Copies of this Offering Memorandum and any supplement hereto (including the pricing supplement) are available free of charge at UBS AG, Prospectus Library, P.O. Box, CH-8098 Zurich (telephone number: +41 44 239 47 03 (voicemail), facsimile: +41 44 239 69 14 or email: [swiss-prospectus@ubs.com](mailto:swiss-prospectus@ubs.com)) and at Santhera Pharmaceuticals Holding AG, Hohenrainstrasse 24, CH-4133 Pratteln (telephone number: +41 61 906 89 50, email: [office@santhera.com](mailto:office@santhera.com)) during regular business hours.

Copies of the Company's financial statements can be downloaded from its website at <http://www.santhera.com/investors-and-media/investor-toolbox/financial-reports>.

For so long as any of the Offered Shares are "restricted securities" within the meaning of Rule 144(a)(3) under the Securities Act, during any period in which the Company is neither subject to the reporting requirements under Sections 13 or 15(d) of the U.S. Securities Exchange Act of 1934, as amended, nor exempt from reporting pursuant to Rule 12g3-2(b) thereunder, the Company will furnish to any holder or beneficial owner of the Offered Shares, or to any prospective purchaser of such Offered Shares designated by any such holder or beneficial owner, the information required to be delivered pursuant to Rule 144A(d)(4) under the Securities Act upon request of any such person. Alternatively, such information can be accessed electronically on the website of the Company at <http://www.santhera.com>.

Information on the Company's website is not part of or incorporated by reference into this Offering Memorandum.

## **NOTICE TO PROSPECTIVE INVESTORS**

No person has been authorized to give any information or to make any representations other than those contained in this Offering Memorandum and, if given or made, such information or representations must not be relied upon as having been authorized.

This Offering Memorandum does not constitute (i) an offer to sell, or a solicitation of an offer to buy any securities other than the Offered Shares; or (ii) an offer to sell, or the solicitation of an offer to buy, such securities by any person in any circumstances in which such offer or solicitation is unlawful.

Neither the delivery of this Offering Memorandum nor any sale made hereunder shall, under any circumstances, create any implication that there has been no change in the affairs of the Company since the date hereof or that the information contained herein is correct as of any time after the date hereof. Any notices containing or announcing amendments or changes to the terms of the Offering or to this Offering Memorandum will be announced through electronic media. Notices required under the Listing Rules will be published on the website of the SIX Swiss Exchange (currently: [https://www.six-group.com/exchanges/news/official\\_notices/search\\_en.html](https://www.six-group.com/exchanges/news/official_notices/search_en.html)). Any such notice will constitute an integral part of this Offering Memorandum.

Prospective investors are advised to familiarize themselves with the entire content of this Offering Memorandum. In making an investment decision, investors must rely on their own investigation of the Company and the terms of the Offering, including the merits and risks involved. Any decision to buy the Offered Shares should be based solely on this Offering Memorandum and any supplement hereto, taking into account that any summary or description set out in this Offering Memorandum of legal provisions, accounting principles or comparison of such principles, corporate structuring or contractual relationships is for information purposes only and should not be considered to be legal, accounting or tax advice or be otherwise relied on. This Offering Memorandum does not contain all the information that would be included in a prospectus for the Offering of the Offered Shares, if such Offering were registered under the Securities Act or made in accordance with the Prospective Directive.

The offer of the Offered Shares to persons resident in jurisdictions other than Switzerland may be affected by the laws of the jurisdiction where recipients of the Offering are resident. No action has been or will be taken in any jurisdiction other than, in the case of the Offered Shares, Switzerland, that would permit a public offering of the Shares or the possession, circulation or distribution of this Offering Memorandum or any other material relating to the Company or Shares in any jurisdiction where action for that purpose is required. Accordingly, the Shares may not be sold, directly or indirectly, and neither this Offering Memorandum nor any other offering material or advertisement in connection with the Shares may be distributed or published, in any form or in any country or jurisdiction except under circumstances that will result in compliance with any applicable laws, rules and regulations of any such country or jurisdiction. Persons resident in countries other than Switzerland should consult their professional advisers as to whether they require any governmental or other consents or need to observe any formalities to enable them to purchase Offered Shares in the Offering.

The Company has represented and agreed that it has not made and will not make any application for listing the Shares on any stock exchange outside Switzerland.

Neither the Company nor the Managers, nor any of their respective representatives, is making any representation to any offeree or purchaser of the Shares regarding the legality of an investment in the Shares by such offeree or purchaser under the laws applicable to such offeree or purchaser. Each investor should consult with his or her own advisers as to the legal, tax, business, financial and related aspects of a purchase of the Shares.

The investors also acknowledge that: (i) they have not relied on the Managers or any person affiliated with the Managers in connection with any investigation of the accuracy of any information contained in this

Offering Memorandum or their investment decision; and (ii) they have relied only on the information contained in this Offering Memorandum, and that no person has been authorized to give any information or to make any representation concerning the Company or the Shares (other than as contained in this Offering Memorandum) and, if given or made, any such other information or representation should not be relied upon as having been authorized by the Company or the Managers.

In connection with the Offering, the Managers and any of their respective affiliates, acting as an investor for its own account, may take up Offered Shares and in that capacity may retain, purchase or sell for its own account such Offered Shares and any Shares or related investments and may offer or sell such Shares or other investments otherwise than in connection with the Offering. Accordingly, references in this Offering Memorandum to the Offered Shares should be read as including any offering or placement of Shares to the Managers or any of their respective affiliates acting in such capacity. The Managers do not intend to disclose the extent of any such investment or transactions otherwise than in accordance with any legal or regulatory obligation to do so. In addition, the Managers or their respective affiliates may enter into financing arrangements (including swaps) with investors in connection with which the Managers (or their respective affiliates) may from time to time acquire, hold or dispose of Shares.

Information on the Company's website, any website directly or indirectly linked thereto or any other website mentioned in this Offering Memorandum is not incorporated by reference into this Offering Memorandum, unless specifically stated herein, and prospective investors should not rely on any such website in making their decision to invest in the Offered Shares.

**A. Notice to United States investors**

THE OFFERED SHARES HAVE NOT BEEN, AND WILL NOT BE, REGISTERED UNDER THE SECURITIES ACT. THE OFFERED SHARES MAY NOT BE OFFERED OR SOLD WITHIN THE UNITED STATES EXCEPT TO QUALIFIED INSTITUTIONAL BUYERS IN RELIANCE ON THE EXEMPTION FROM REGISTRATION PROVIDED BY RULE 144A AND OUTSIDE THE UNITED STATES IN OFFSHORE TRANSACTIONS IN RELIANCE ON REGULATION S. EACH PROSPECTIVE PURCHASER OF THE OFFERED SHARES IS HEREBY NOTIFIED THAT SELLERS MAY BE RELYING ON THE EXEMPTION FROM THE REGISTRATION REQUIREMENTS OF SECTION 5 OF THE SECURITIES ACT PROVIDED BY RULE 144A. THIS OFFERING MEMORANDUM DOES NOT CONSTITUTE A PROSPECTUS WITHIN THE MEANING OF SECTION 10 OF THE SECURITIES ACT.

THE OFFERED SHARES ARE SUBJECT TO RESTRICTIONS ON TRANSFERABILITY AND RE-SALE AND MAY NOT BE TRANSFERRED OR RESOLD EXCEPT AS PERMITTED UNDER THE SECURITIES ACT AND THE APPLICABLE SECURITIES LAWS OF ANY OTHER JURISDICTION. SEE "SELLING AND TRANSFER RESTRICTIONS" BEGINNING ON PAGE 151. PROSPECTIVE PURCHASERS SHOULD BE AWARE THAT THEY MAY BE REQUIRED TO BEAR THE FINANCIAL RISKS OF THIS INVESTMENT FOR AN INDEFINITE PERIOD OF TIME.

THE OFFERED SHARES HAVE NOT BEEN APPROVED OR DISAPPROVED BY THE U.S. SECURITIES AND EXCHANGE COMMISSION OR ANY STATE SECURITIES COMMISSION IN THE UNITED STATES OR ANY OTHER U.S. REGULATORY AUTHORITY, NOR HAVE ANY OF THE FOREGOING AUTHORITIES PASSED UPON OR ENDORSED THE MERITS OF THIS OFFERING OR THE ACCURACY OR ADEQUACY OF THIS OFFERING MEMORANDUM. ANY REPRESENTATION TO THE CONTRARY MAY BE A CRIMINAL OFFENSE IN THE UNITED STATES.

EACH PURCHASER WILL BE DEEMED TO HAVE ACKNOWLEDGED, REPRESENTED AND WARRANTED THAT IT UNDERSTANDS AND AGREES TO THE FOREGOING.

## **B. Notice to European Economic Area investors**

In relation to each Member State of the European Economic Area that has implemented the Prospectus Directive (each, a “**Relevant Member State**”), with effect from and including the date on which the Prospectus Directive is implemented in that Relevant Member State (the “**Relevant Implementation Date**”) an offer to the public of any Shares that are the subject of the Offering contemplated by this Offering Memorandum may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State may be made at any time, with effect from and including the Relevant Implementation Date under the following exemptions under the Prospectus Directive:

- (i) to any legal entity that is a qualified investor as defined in the Prospectus Directive; or
- (ii) in any other circumstances falling within article 3(2) of the Prospectus Directive,

provided that no such offer of Shares shall require the Company or the Managers to publish a prospectus pursuant to article 3 of the Prospectus Directive or supplement a prospectus pursuant to article 16 of the Prospectus Directive.

For the purposes of this provision, the expression an “**offer to the public**” in relation to any Shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the Shares to be offered so as to enable an investor to decide to purchase or subscribe the Shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State and the expression “**Prospectus Directive**” means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive), and includes any relevant implementing measure in each Relevant Member State and the expression “**2010 PD Amending Directive**” means Directive 2010/73/EU.

## **C. Notice to United Kingdom investors**

This Offering Memorandum is only directed at, and will only be provided to, persons to whom interests may lawfully be promoted pursuant to section 21 of the Financial Services and Markets Act 2000 (“**FSMA**”). In particular, this Offering Memorandum is only directed at, and will only be provided to, investment professionals within the meaning of article 19 of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (“**FPO**”) (“**Relevant Persons**”). Any investment or investment activity to which this Offering Memorandum relates is available only to Relevant Persons and dealings hereunder will be made only with Relevant Persons. Persons who are not investment professionals within the meaning of article 19 of the FPO should not rely on this Offering Memorandum.

This Offering Memorandum has not been delivered for approval to the Financial Conduct Authority (“**FCA**”) in the United Kingdom or to an authorized person within the meaning of FSMA. No approved prospectus within the meaning of section 85 of FSMA or of the Prospectus Directive has been published or is intended to be published in relation to the Offering. This Offering Memorandum does not constitute a prospectus for the purposes of FSMA or the Prospectus Directive. As used herein, “**United Kingdom**” means the United Kingdom of Great Britain and Northern Ireland.

## **D. Notice to Australian investors**

This Offering Memorandum and the Offering is only made available in Australia to persons to whom a disclosure document is not required to be given under Chapter 6D of the Corporations Act 2001. This Offering Memorandum is not a prospectus, product disclosure statement or any other form of formal “disclosure document” for the purposes of the Corporations Act, and is not required to, and does not, contain all the information that would be required in a disclosure document under the Corporations Act. If you are in Australia, this document is made available to you provided you are a person to whom an offer of securities can be made without a disclosure document such as a professional investor or sophisticated investor for the purposes of Chapter 6D of the Corporations Act.

This Offering Memorandum has not been, and will not be, lodged with the Australian Securities and Investments Commission (“ASIC”) as a disclosure document for the purpose of the Corporations Act 2001. No Shares may be offered for sale (or transferred, assigned or otherwise alienated) to investors in Australia for at least 12 months after this issue, except in circumstances where disclosure to investors is not required under Chapter 6D of the Corporations Act 2001 or unless a disclosure document that complies with the Corporations Act 2001 is lodged with the ASIC. Each investor acknowledges the above and, by applying for Shares under this Offering Memorandum, gives an undertaking not to sell those Shares (except in the circumstances referred to above) for 12 months after their issue.

The persons referred to in this Offering Memorandum may not hold Australian financial services licenses and may not be licensed to provide financial product advice in relation to the Shares. No “cooling-off” regime will apply to an acquisition of any interest in the Company.

This Offering Memorandum does not take into account the investment objectives, financial situation or needs of any particular person. Accordingly, before making any investment decision in relation to this Offering Memorandum, you should assess whether the acquisition of any interest in the Company is appropriate in light of your own financial circumstances or seek professional advice.

#### **E. Notice to Japanese investors**

The Shares have not been and will not be registered under the Financial Instruments and Exchange Law, as amended (the “FIEL”). This Offering Memorandum is not an offer of Shares for sale, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means any person resident in Japan, including any corporation or entity organized under the laws of Japan) or to others for reoffer or resale, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan, except pursuant to an exemption from the registration requirements under the FIEL and otherwise in compliance with such law and any other applicable laws, regulations and ministerial guidelines of Japan.

#### **F. Notice to Canadian investors**

The Offered Shares may not, directly or indirectly, be offered, sold or distributed within Canada, or to, or for the benefit or account of, any resident of Canada, except in compliance with all applicable securities laws, regulations or rules of the provinces and territories of Canada and with the prior approval of the Global Coordinator and Bookrunner, acting on behalf of the Managers. This Offering Memorandum, or any other material relating to the Offered Shares, may not be distributed or delivered in Canada, except in compliance with all applicable securities laws, regulations or rules of the provinces and territories of Canada. No securities commission or similar authority in Canada has reviewed or in any way passed upon this Offering Memorandum or the merits of the Offered Shares, and any representation to the contrary is an offence.

The Company is not a reporting issuer in any province or territory of Canada and all of its executive management and directors are ordinarily resident outside of Canada. The Offered Shares are being sold primarily outside Canada and may be sold in Canada only to purchasers resident or located in the Provinces of Ontario, Quebec, Alberta and British Columbia (the “Canadian Jurisdictions”), purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions (“NI 45-106”) or the Securities Act (Ontario) (the “OSA”), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations (“NI 31-103”). Any resale of the Offered Shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable Canadian securities laws and purchasers in the Canadian Jurisdictions should consult with a legal advisor prior to any resale of the Offered Shares whether in Canada or elsewhere.

Each purchaser of Offered Shares in the Canadian Jurisdictions will be deemed to have represented and agreed as follows:

- (a) The purchaser is purchasing, or is deemed to be purchasing, the Offered Shares as principal for investment purposes and not with a view to resale or further distribution.
- (b) The purchaser is not an individual and is resident in one of the Canadian Jurisdictions.
- (c) The purchaser is an accredited investor as defined in NI 45-106 and the OSA (other than a person that was created or is used solely to purchase or hold securities as an accredited investor).
- (d) The purchaser is a permitted client as defined in NI 31-103.
- (e) The purchaser will provide all information and documentation reasonably requested by the Company or the Global Coordinator and Bookrunner, acting on behalf of the Managers, to establish that the purchaser is an accredited investor (and the applicable paragraph number in the definition thereof) and a permitted client, and to permit them to complete any reports required to be filed in any Canadian Jurisdiction.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this Offering Memorandum (including any amendment hereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts ("NI 33-105"), the Managers are relying on the exemption therein from the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with the Offering.

In connection with the subscription for the Offered Shares, the purchaser has required that all documents relating thereto be drawn up in the English language only. Dans le cadre de la souscription pour des actions offertes, l'acquéreur a requis que tous les documents s'y rattachant soient rédigés en anglais seulement.

#### **G. General sales restrictions**

No action has been or will be taken by the Company or the Managers in any jurisdiction other than, with respect to the Offered Shares, Switzerland, that would, or is intended to, permit a public offering of the Shares, or possession or distribution of this Offering Memorandum or any other offering material, in any country or jurisdiction where further action for that purpose is required.

## **SERVICE OF PROCESS AND ENFORCEMENT OF CIVIL LIABILITIES UNDER U.S. LAW**

The Company is a stock corporation organized under Swiss law. Most of its assets are located outside the U.S. In addition, none of its directors and executive officers are residents of the U.S. and all of or a substantial portion of their assets are located outside the U.S. As a result, it may not be possible for prospective investors to effect service of process within the United States upon those persons or the Company, or to enforce against them judgements of U.S. courts based upon the civil liability provisions of the federal securities laws of the United States. Furthermore, there is doubt as to the enforceability in Switzerland in original actions or in actions for enforcement of judgements of U.S. courts of liabilities based solely upon the federal securities laws of the United States. See also risk factor “*U.S. shareholders may not be able to obtain judgments or enforce civil liabilities against the Company or its directors or executive officers.*” beginning on page 35.

## THIRD-PARTY DATA

Information contained in this Offering Memorandum relating to diseases, the mode of action of our compounds, treatment options, patient prevalence, competitor approaches, and products or product candidates developed or marketed by third parties was derived either directly from the public domain (including scientific journals) or from estimates made by the Company based on its own, third-party, or publicly available data.

The Company has not independently verified any third-party data and cannot assure prospective investors of the accuracy or completeness of, and takes no responsibility for, such data. While the Company believes the estimates from which such data was derived to be reasonable, it cannot assure prospective investors as to their accuracy or that another third-party using different methods to assemble, analyze or compute relevant data or information would obtain the same result. The Company does not intend, and does not assume any obligation, to update third-party data set forth in this Offering Memorandum, except as required by law. Finally, prospective investors should be aware that data in this Offering Memorandum and estimates based on that data may be unreliable indicators of future results.

## FORWARD-LOOKING STATEMENTS

This Offering Memorandum contains statements that are, or may be deemed to be, forward-looking statements. In some cases, these forward-looking statements can be identified by the use of forward-looking terminology or subjective assessments, including the words “*aims*”, “*believes*”, “*estimates*”, “*anticipates*”, “*expects*”, “*targets*”, “*intends*”, “*may*”, “*will*”, “*plans*”, “*continue*”, “*projects*”, “*predicts*”, “*assumes*”, “*could*” or “*should*” or, in each case, their negative or other variations or comparable terminology or by discussions of strategies, plans, objectives, targets, goals, future events or intentions. These forward-looking statements include matters that are not historical facts or that may not otherwise be provable by reference to past events, and are based on assumptions regarding the Company’s present and future business strategies and the environment in which it operates and will operate in the future. They include statements regarding the Company’s intentions, beliefs or current expectations. By their nature, forward-looking statements involve known and unknown risks and uncertainties because they relate to events and/or depend on circumstances that may or may not occur in the future. Forward-looking statements are not guarantees of future performance and may prove to be erroneous or unfounded in the future. Prospective investors should not place undue reliance on these forward-looking statements. The risks and uncertainties facing the Company that could affect the future accuracy of these forward-looking statements include, but are not limited to, the factors discussed under “*Risk Factors*” beginning on page 11 and elsewhere.

The risks described under “*Risk Factors*” beginning on page 11 are not exhaustive. Other sections of this Offering Memorandum describe additional factors that may adversely affect the Company’s results of operations, financial condition, liquidity, dividend policy and the development of the markets in which it operates. The Company urges prospective investors to read the sections of this Offering Memorandum titled “*Risk Factors*”, “*Management’s Discussion and Analysis of Financial Condition and Results of Operations*”, “*The Company and its Business*” and “*Legal and Regulatory Environment*” beginning on pages 11, 43, 65 and 97, respectively, for a more complete discussion of the factors that could affect its future performance and the industry in which it operates.

Any forward-looking statements are only made as of the date of this Offering Memorandum and the Company does not intend, and does not assume any obligation, to update any forward-looking statements contained in this Offering Memorandum, except as required by Swiss law or applicable stock exchange regulations. New risks may emerge from time to time, and it is not possible for the Company to predict all such risks, nor can it assess the impact of all such risks on its business or the extent to which any risks, or combination of risks and other factors, may cause actual results to differ materially from those contained in any forward-looking statements. Given these risks and uncertainties, prospective investors should not rely on forward-looking statements as a prediction of actual performance or results.

Many factors may cause the Company's results of operations, financial condition, liquidity, dividend policy and the development of the markets in which it operates to differ materially from those expressed or implied by the forward-looking statements contained in this Offering Memorandum and thereby adversely affect the achievement of the Company's financial targets.

## **PRESENTATION OF FINANCIAL AND OTHER INFORMATION**

This Offering Memorandum contains:

- audited consolidated financial statements (including the notes thereto) of the Company as of and for the year ended December 31, 2015 (the “**2015 Consolidated Financial Statements**”) and audited statutory financial statements (including the notes thereto) of the Company as of and for the same year (the “**2015 Statutory Financial Statements**”), and the auditors' reports thereon, all as part of the Company's annual report for the same year;
- audited consolidated financial statements (including the notes thereto) of the Company as of and for the year ended December 31, 2016 (the “**2016 Consolidated Financial Statements**”) and audited statutory financial statements (including the notes thereto) of the Company as of and for the same year (the “**2016 Statutory Financial Statements**”), and the auditors' reports thereon, all as part of the Company's annual report for the same year;
- audited consolidated financial statements (including the notes thereto) of the Company as of and for the year ended December 31, 2017 (the “**2017 Consolidated Financial Statements**”) and audited statutory financial statements (including the notes thereto) of the Company as of and for the same year (the “**2017 Statutory Financial Statements**”), and the auditors' reports thereon, all as part of the Company's annual report for the same year; and
- interim condensed consolidated financial statements (including the notes thereto) of the Company as of and for the nine months ended September 30, 2018 (the “**Unaudited Interim Condensed Consolidated Financial Statements**”) and unaudited interim statutory financial statements (including the notes thereto) of the Company as of and for the nine months ended September 30, 2018 (the “**Unaudited Interim Statutory Financial Statements**”).

The 2015 Consolidated Financial Statements, the 2016 Consolidated Financial Statements and the 2017 Consolidated Financial Statements (collectively the “**Audited Consolidated Financial Statements**”) are presented in Swiss francs and have been prepared in accordance with International Financial Reporting Standards as issued by the International Accounting Standards Board (“**IFRS**”). The Unaudited Interim Condensed Consolidated Financial Statements are presented in Swiss francs and have been prepared in accordance with International Accounting Standard 34 (“**IAS 34**”). For a further description of the Company's accounting policies, see Note 2 to the Audited Consolidated Financial Statements and the Unaudited Interim Condensed Consolidated Financial Statements, respectively.

The 2015 Statutory Financial Statements, the 2016 Statutory Financial Statements, the 2017 Statutory Financial Statements and the Unaudited Interim Statutory Financial Statements (collectively the “**Statutory Financial Statements**”) are presented in Swiss francs and have been prepared in accordance with the provisions governing the preparation of financial statements of Swiss law.

The Audited Consolidated Financial Statements have been audited by Ernst & Young AG, Basel (“**EY**”), in accordance with Swiss Auditing Standards and International Standards on Auditing (ISA), and the Statutory Financial Statements have been audited by EY in accordance with Swiss law and Swiss Auditing Standards, as stated in their reports.

Historical financial information relating to the Company's results, financial position and cash flows presented in this Offering Memorandum, unless stated otherwise, has been extracted or derived from the Audited Consolidated Financial Statements and the Unaudited Interim Condensed Consolidated Financial Statements.

Investors should be aware that the accounting requirements of IFRS and the CO differ in certain respects from each other and from generally accepted accounting principles in certain other countries, including generally accepted accounting principles in the United States (“U.S. GAAP”). Therefore, the financial information contained herein that is prepared in accordance with either IFRS or the CO is not comparable with each other or such other generally accepted accounting principles, including U.S. GAAP. In addition, investors should be aware that the future financial performance of the Company may vary substantially from its historic financial performance.

Certain figures contained in this Offering Memorandum, including financial information, have been subject to rounding adjustments. Accordingly, in certain instances, the amounts shown as totals in tables or elsewhere may not conform exactly to the arithmetic total figures that precede them. In addition, certain percentages in the Offering Memorandum reflect calculations based upon the underlying information prior to rounding and, accordingly, may not conform exactly to the percentages that would be derived if the relevant calculations were based upon the rounded numbers.

## EXCHANGE RATE INFORMATION

In this Offering Memorandum, references to “CHF” or “Swiss francs” are to the lawful currency of Switzerland, references to “EUR” or “euros” are to the lawful currency of the European Union, and references to “USD” are to the lawful currency of the United States. Except as otherwise stated, amounts appearing in this Offering Memorandum that were converted into CHF from other currencies were converted in accordance with the principles described in the Audited Consolidated Financial Statements. References to the “U.S.,” “USA” or the “United States” are to the United States of America.

The Group’s business is conducted primarily in Switzerland, the eurozone, the United Kingdom and the U.S. The Company maintains its books and records in, and prepares its financial statements in, CHF. The majority of the Group’s sales are generated in EUR. The majority of the Group’s expenses from its business activities are currently denominated in CHF, EUR and USD. Except as otherwise stated, amounts appearing in this Offering Memorandum that were converted into CHF from other currencies were converted in accordance with the principles described in the Audited Consolidated Financial Statements.

The following tables set forth, for the periods indicated, the average, high, low and period-end EUR/CHF and USD/CHF reference exchange rates. No representation is made that Swiss francs could have been, or could be, converted into euros or U.S. dollars, as applicable, at these rates. The Bloomberg Composite Rate of the euro on December 7, 2018 was EUR 0.8850 per CHF 1.00. The Bloomberg Composite Rate of the U.S. dollar on December 7, 2018 was USD 1.0084 per CHF 1.00.

### Euro per Swiss franc

<b>Year</b>	<b>Average</b>	<b>High</b>	<b>Low</b>	<b>Period-End</b>
2013 .....	0.8125	0.8276	0.7949	0.8150
2014 .....	0.8234	0.8328	0.8078	0.8313
2015 .....	0.9374	1.0253	0.8320	0.9185
2016 .....	0.9174	0.9359	0.8966	0.9326
2017 .....	0.9006	0.9402	0.8516	0.8544
<b>Month</b>	<b>Average</b>	<b>High</b>	<b>Low</b>	<b>Period-End</b>
June 2018 .....	0.8650	0.8688	0.8607	0.8633
July 2018 .....	0.8605	0.8659	0.8543	0.8633
August 2018 .....	0.8767	0.8891	0.8642	0.8891
September 2018 .....	0.8856	0.8924	0.8762	0.8820
October 2018 .....	0.8759	0.8797	0.8708	0.8764
November 2018 .....	0.8794	0.8866	0.8727	0.8849
December 2018 (through December 7, 2018) .....	0.8838	0.8850	0.8825	0.8850

*Source:* For the indicated periods, the table shows the high, low, average and period-end Bloomberg Composite Rate expressed as euro per CHF 1.00. The Bloomberg Composite Rate is a “best market” calculation,

in which, at any point in time, the bid rate is equal to the highest bid rate of all contributing bank indications and the ask rate is set to the lowest ask rate offered by these banks. The Bloomberg Composite Rate is a mid-value rate between the applied highest bid rate and the lowest ask rate.

#### USD per Swiss franc

Year	Average	High	Low	Period-End
2013 .....	1.0794	1.1300	1.0221	1.1200
2014 .....	1.0935	1.1464	1.0057	1.0057
2015 .....	1.0401	1.1925	0.9712	0.9976
2016 .....	1.0155	1.0485	0.9709	0.9814
2017 .....	1.0159	1.0571	0.9754	1.0271
Month	Average	High	Low	Period-End
June 2018 .....	1.0100	1.0185	1.0028	1.0081
July 2018 .....	1.0053	1.0121	0.9973	1.0105
August 2018 .....	1.0123	1.0307	1.0035	1.0307
September 2018 .....	1.0328	1.0425	1.0228	1.0242
October 2018 .....	1.0059	1.0163	0.9909	0.9909
November 2018 .....	0.9992	1.0075	0.9910	1.0012
December 2018 (through December 7, 2018) .....	1.0044	1.0084	1.0019	1.0084

*Source:* For the indicated periods, the table shows the high, low, average and period-end Bloomberg Composite Rate expressed as U.S. dollars per CHF 1.00. The Bloomberg Composite Rate is a “best market” calculation, in which, at any point in time, the bid rate is equal to the highest bid rate of all contributing bank indications and the ask rate is set to the lowest ask rate offered by these banks. The Bloomberg Composite Rate is a mid-value rate between the applied highest bid rate and the lowest ask rate.

## SHARE PRICE INFORMATION

The following tables set forth, for the periods indicated, the high and low market prices of the Company’s shares on the SIX Swiss Exchange.

#### In Swiss francs

Year	High	Low
2013 .....	5.80	1.34
2014 .....	100.10	3.55
2015 .....	134.40	82.60
2016 .....	89.45	39.50
2017 .....	82.00	25.10
Quarter ended	High	Low
March 31, 2016 .....	91.25	55.00
June 30, 2016 .....	86.35	63.80
September 30, 2016 .....	84.50	48.50
December 31, 2016 .....	59.90	37.05
March 31, 2017 .....	82.00	53.30
June 30, 2017 .....	80.20	55.50
September 30, 2017 .....	74.00	29.10
December 31, 2017 .....	40.30	25.10
March 31, 2018 .....	41.15	16.84
June 30, 2018 .....	19.90	15.90
September 30, 2018 .....	20.20	14.52
Month	High	Low
June 2018 .....	18.20	15.90
July 2018 .....	20.20	16.08

August 2018.....	17.58	16.30
September 2018 .....	17.26	14.52
October 2018.....	16.80	14.50
November 2018.....	16.38	11.12
December 2018 (through December 7, 2018).....	11.78	10.00

## I. SUMMARY

*The following summary is not intended to be complete and is to be read together with the more detailed information set out elsewhere in this Offering Memorandum. In particular, investors should consult the sections “The Company and its Business” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” beginning on pages 65 and 43, respectively, and should carefully consider the information presented in the section titled “Risk Factors” beginning on page 11 before making an investment decision.*

We are a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. The portfolio comprises clinical stage treatment and a marketed treatment for neuro-ophthalmologic, neuromuscular and pulmonary diseases. We believe that we have developed significant expertise in the understanding of the underlying causes of these diseases and our goal is to become a market leader in the development and commercialization of products for their treatment.

Our lead compound is idebenone, an anti-oxidant agent that we believe can help improve production of energy by mitochondria in certain patients whose mitochondrial function is impaired by genetic defects. Idebenone is the active ingredient in Raxone<sup>®</sup>, our product marketed in the EU and Israel for the treatment of Leber’s hereditary optic neuropathy (“**LHON**”), and a potential active ingredient or product candidate for the treatment of various mitochondrial, neuromuscular and neuro-ophthalmological diseases. Idebenone is a drug that has been well-known for decades, with a well-known mode of action and a well-established safety profile. Idebenone’s mode of action suggests the potential of a wide therapeutic application.

In November 2018, we entered into an agreement (the “**Option Agreement**”) with Idorsia Pharmaceuticals Ltd (“**Idorsia**”) under which we have acquired from Idorsia an option for the exclusive sub-license relating to commercialize vamorolone, a non-hormonal steroid modulator developed by ReveraGen BioPharma, Inc. (“**ReveraGen**”), in all indications and all territories except Japan and South Korea (for which Idorsia has the right under the Option Agreement, in its sole discretion, to grant us a sublicense). As initial consideration for the acquisition of the option from Idorsia, we issued the 1,000,000 Idorsia Shares to Idorsia and have agreed to pay USD 20.0 million in cash from the proceeds of this Offering. Both the issuance of the 1,000,000 Idorsia Shares to Idorsia and the payment of the USD 20.0 million in cash are non-refundable. The cash amount is intended to compensate Idorsia for having already paid USD 15.0 million to ReveraGen to fund the ongoing Phase IIb trial in patients with DMD. We will be able to exercise this option against payment of USD 30.0 million at the latest when the data from the Phase IIb study in Duchenne muscular dystrophy (“**DMD**”) patients are available, which is expected to be the case in 2020 according to the current development plan. ReveraGen has granted its consent to the execution of the Option Agreement.

Currently, we are focusing our development and commercialization efforts on the following product and product candidates:

- *Our marketed product: Raxone<sup>®</sup> in LHON.* LHON is a rare, severe hereditary eye disease affecting primarily men in their 20s and 30s and leading to central vision loss in both eyes. Raxone<sup>®</sup> is, to our knowledge, the first product approved for the treatment of LHON. We received marketing authorization for Raxone<sup>®</sup> in LHON in all 28 EU countries, Norway, Iceland and Liechtenstein in September 2015, and our exclusive distributor received marketing authorization for it in Israel in August 2017. In addition, we filed a marketing authorization application (“**MAA**”) for Raxone<sup>®</sup> in LHON in South Korea, which was accepted for review in June 2018.
- *Our lead product candidate: Raxone<sup>®</sup> in DMD.* DMD is one of the most common types of inherited degenerative muscle weakness. With symptoms starting at young age, patients commonly are unable to walk by their teenage years and require mechanical ventilation to prolong survival beyond their late teenage years. In the EU, we filed an MAA with the European Medicines Agency (“**EMA**”) for Raxone<sup>®</sup> in certain patients with DMD with declining respiratory function who are not receiving steroids. However, the EMA’s Committee for Medicinal Products for Human Use issued a negative

opinion on our MAA, *i.e.*, it recommended that the European Commission not grant conditional marketing authorization for the treatment of DMD in September 2017 and maintained such negative opinion in January 2018 after a re-examination procedure. Such negative opinion does not impact the marketing authorization of Raxone<sup>®</sup> for the treatment of LHON. In July 2018, we announced results of a comparative analysis of the Phase III DELOS clinical trial outcome and new data from natural history studies. This analysis showed that the treatment effect with idebenone observed in the Phase III DELOS clinical trial can be linked to a delay in the initiation of assisted ventilation by three years, which is of high clinical relevance. We and our academic partners intend to prepare for the publication of additional clinical data relating to the long-term efficacy of idebenone on respiratory function outcomes in patients with DMD, supporting the positive data from the successful Phase III DELOS clinical trial. We plan to discuss the findings with regulators in the coming months and to include them in the regulatory dossier in preparation of MAAs for idebenone in DMD in Europe and the U.S. in 2019.

- In the U.S., we received fast track designation for Raxone<sup>®</sup> in DMD from the U.S. Food & Drug Administration (“**FDA**”). In addition, we are currently conducting a phase III clinical trial with certain DMD patients with declining respiratory function who are receiving steroids and we currently expect top line data from this trial in 2020.
- *Vamorolone in DMD*. If and when we exercise our option pursuant to the Option Agreement with Idorsia, we intend to develop vamorolone for early stage DMD patients requiring an anti-inflammatory, muscle strengthening glucocorticoid before onset of respiratory decline. We expect the combination of vamorolone and idebenone to address the medical needs of DMD patients at all disease stages. ReveraGen has conducted extensive non-clinical studies, Phase Ia and Ib studies and Phase IIa and IIa-extension studies of vamorolone. Based on knowledge obtained from these studies, ReveraGen is currently conducting the Phase IIb - VISION DMD trial, which together with the previous studies could form the basis for approval of vamorolone in DMD.
- *Our early stage product candidate, omigapil in congenital muscular dystrophy (“CMD”)*. CMD is a group of inherited conditions that causes progressive and potentially life-threatening loss of muscle tissue, affecting frequently newborns and children. We are exploring the compound omigapil for the treatment of CMD in children and adolescents. In April 2018, we announced that the top line data of a phase I clinical trial for omigapil in CMD (CALLISTO) that we conducted in collaboration with the National Institute of Neurological Disease and Strokes, an institute within the National Institutes of Health in the U.S., suggest that the trial met its primary objective to establish a favorable pharmacokinetic profile of omigapil and demonstrated that the drug was safe and well tolerated in the children and adolescents that participated in the trial. Further development is currently being discussed with clinical experts and regulators.
- *Our early stage product candidate, POL6014 in cystic fibrosis (“CF”)*. CF is a rare, life-threatening, progressive genetic disease that is typically diagnosed in young children and affects primarily the lungs but also the digestive system. In February 2018, we in-licensed the compound POL6014 that we believe has the potential to treat CF and other neutrophilic lung diseases. Based on prior development work by Polyphor, including two phase I clinical trials, we have started a phase I multiple ascending dose clinical trial of POL6014 in CF in the fourth quarter of 2018.

We have entered into a number of strategic development collaborations, by in-licensing and co-developing promising product candidates, and established commercial relationships with distributors to exploit the commercial potential of our products. We believe that these collaborations demonstrate our ability to successfully partner with global pharmaceutical and biotechnology companies and to establish ourselves as an attractive development and commercialization ally.

We are managed by a capable, experienced and professional team, with the members of our Executive Management alone having more than 80 years of combined pharmaceutical industry experience. See “*Board of Directors and Executive Management*” beginning on page 122.

Santhera in its present form was founded in September 2004. The Shares have been listed on the SIX Swiss Exchange since November 2006. Our headquarters are located in Pratteln, Switzerland, with subsidiaries in Switzerland, Germany, the United Kingdom, Italy, the Netherlands, Spain, Liechtenstein, the U.S., Canada and Finland. As of September 30, 2018, we had 119 employees (113.6 full-time equivalent). These figures are expected to increase in the near future due to our ongoing expansion of operations relating to the commercialization of Raxone®.

## II. SUMMARY OF THE TERMS OF THE OFFERING

<b>Offering</b>	This Offering relates to the Offered Shares only. The Idorsia Shares have already been sold and are not being offered pursuant to this Offering Memorandum. This Offering consists of (i) a public offering in Switzerland, (ii) private placements in certain jurisdictions outside the United States and Switzerland in accordance with applicable securities laws and in reliance on Regulation S, and on the basis of exemptions provided by the Prospectus Directive, and (iii) private placements within the United States to QIBs in reliance on Rule 144A.
<b>Shares</b>	The Shares are fully paid-in registered shares ( <i>Namenaktien</i> ) of the Company with a nominal value of CHF 1.00 each. See “— <i>Form of Shares</i> ” in “ <i>Offering and Sale</i> ” beginning on page 149.
<b>Issued Shares before Issuance of the New Shares</b>	6,527,479 Shares (reflects the number of Shares prior to the issuance of the Idorsia Shares on November 21, 2018 and the issuance of any Offered Shares).
<b>Idorsia Shares</b>	1,000,000 Shares issued out of the Company’s authorized share capital, under exclusion of the preemptive rights of the existing shareholders, in a private placement to Idorsia on November 21, 2018 as equity consideration for the acquisition from Idorsia of an option for the exclusive sub-license relating to ReveraGen’s vamorolone. See “— <i>License and collaboration agreements</i> ” in “ <i>The Company and its Business</i> ” beginning on page 84. The Idorsia Shares do not form part of the Offering.
<b>Percentage of Share Capital represented by Idorsia Shares</b>	<p>The 1,000,000 Idorsia Shares represent 15.32% of the Company’s share capital recorded in the commercial register immediately prior to the issuance of the Idorsia Shares.</p> <p>Immediately after the issuance of the Idorsia Shares, the share capital of the Company consisted of 7,527,479 Shares (including the Idorsia Shares). The 1,000,000 Idorsia Shares represented 13.28% of the Company’s share capital recorded in the commercial register immediately after their issuance (these percentages do not take into consideration the up to 5,000,000 Offered Shares which have been resolved to be created at the extraordinary shareholders' meeting of the Company held on December 11, 2018).</p>
<b>Offered Shares</b>	The up to 5,000,000 Offered Shares have been resolved to be created at the extraordinary shareholders' meeting of the Company held on December 11, 2018.
<b>Use of Proceeds</b>	The Company targets gross proceeds of approximately CHF 50 million from the sale of up to 5,000,000 new Shares offered in the Offering (based on the maximum number of Offered Shares and the closing price of the Shares on the SIX Swiss Exchange on December 11, 2018), which would result in approximately CHF 46.0 million in net proceeds after deducting estimated underwriting commissions, estimated offering expenses payable by the Company and the Swiss federal issue stamp duty ( <i>Emissionsabgabe</i> ). The Company intends to use the net proceeds from the sale of the Offered Shares to fund the USD 20.0 million cash component of the consideration for the acquisition of the option for the exclusive sub-license relating

to ReveraGen's vamorolone and any net proceeds from the Offering in excess of that amount for general corporate purposes, as further described in this Offering Memorandum. See "Use of Proceeds" beginning on page 37. The Company intends to complete the Offering as long as the net proceeds exceed the CHF equivalent of USD 20 million.

**Percentage of Share Capital represented by Offered Shares**

Assuming the maximum of 5,000,000 Offered Shares will be sold, they will represent 66.42% of the Company's share capital recorded in the commercial register immediately prior to the issuance of the Offered Shares and 39.91% of the Company's share capital recorded in the commercial register immediately after to the issuance of the Offered Shares (these percentages take into consideration the 1,000,000 Idorsia Shares).

**Outstanding Shares after issuance of the Idorsia Shares and completion of the Offering**

After giving effect to the issuance of the Idorsia Shares and upon completion of the Offering and assuming that all 5,000,000 Offered Shares are sold in the Offering, the share capital of the Company will consist of 12,527,479 Shares.

**Treasury Shares**

As of the date of this Offering Memorandum, the Company holds 51,996 treasury Shares.

**Offer Price and final number of Offered Shares**

The Company expects to determine the Offer Price and the final number of Offered Shares together with the Global Coordinator and Bookrunner, acting on behalf of the Managers, following an accelerated bookbuilding process on or around December 14, 2018.

The Offer Price and the final number of Offered Shares sold in the Offering will be published in the electronic media, by press release and in a pricing supplement to this Offering Memorandum on or around December 14, 2018.

**Offer Period**

The offer period is expected to be from December 12, 2018, to December 13, 2018, at 4:00 p.m. (CET).

The Company, together with the Global Coordinator and Bookrunner, acting on behalf of the Managers, reserves the right to extend or shorten the offer period or terminate the Offering, without any prior notice, at any time and for any reason.

**Lock-up**

The Company has agreed with the Managers that, during the period commencing on the date of the Accelerated Book-Build Agreement and ending 90 days after the First Day of Trading, and subject to certain exceptions, the Company and its subsidiaries shall not, without the prior written consent of the Global Coordinator and Bookrunner, acting on behalf of the Managers, (i) issue, offer, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, pledge, grant instruction rights (*Weisungsrechte*) pursuant to art. 25 FISA or otherwise transfer or dispose of (or publicly announce any such issuance, offer, sale or disposal), directly or indirectly, or file a registration statement under any securities regulation relating to, any Shares or any securities representing or convertible into or exchangeable or exercisable for Shares or warrants or other rights to purchase any Shares, (ii) enter into any swap, hedge or other arrangement that transfers to another, in whole or in part,

any of the economic consequences of ownership of the Shares, or (iii) announce its intention to do any of the foregoing whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Shares or other securities, in cash or otherwise. The foregoing lock-up obligation shall not apply to (i) any options, Shares, stock appreciation rights, performance shares, restricted share units or other equity-linked instruments granted to (or for the benefit of) the Group's employees, management, directors and advisors pursuant to its existing participation or similar plans (the "**Plans**"), (ii) Shares issued upon the exercise of awards granted pursuant to such Plans, issued to a subsidiary of the Company to serve as reserve for such exercises, or issued upon the exercise of conversion rights under the Company's CHF 60 million Senior Unsecured Convertible Bonds 2017-2022, (iii) Shares issued or rights to receive Shares granted as consideration or in-licensing of compounds, product candidates, technology or businesses, provided that the market value of such Shares (or the Shares underlying such rights, as applicable) does not exceed CHF 30 million in aggregate and the acquiring entity enters into the same lock-up undertaking with the Managers, (iv) Shares or other securities of the Issuer acquired in open market transactions after the First Day of Trading, (v) transactions among Group companies, provided that the Company shall give prior notice to the Global Coordinator and Bookrunner, acting on behalf of the Managers, and any Group company acquiring Shares shall enter into the same lock-up undertaking or (vi) any corporate actions in connection with a takeover offer, capital reorganization, legal merger, corporate restructuring, split-up or similar transaction or process, in each case involving the Company.

The Idorsia Shares are subject to a lock-up undertaking expiring if and when vamorolone receives marketing authorization in DMD in the United States.

**Dividends and Dividend Policy**

See "*Dividends and Other Distributions*" beginning on page 38.

**Voting Rights**

Each Share carries one vote. See "*Description of the Company's Capital Structure and Shares*" beginning on page 133.

**Listing and Trading**

Application has been made and approval has been given subject to certain conditions to list the New Shares on the SIX Swiss Exchange according to the International Reporting Standard.

The Idorsia Shares were listed on the SIX Swiss Exchange according to the International Reporting Standard on November 22, 2018.

The Company expects that the Offered Shares will be listed on the SIX Swiss Exchange according to the International Reporting Standard on or around December 18, 2018.

**Risk Factors**

For a review of certain considerations that investors should take into account in deciding whether to purchase any Shares see "*Risk Factors*" beginning on page 11.

**Payment and Settlement**

The Shares are cleared through SIS. It is expected that delivery of the Offered Shares against payment of the Offer Price will be made through the facilities of SIS on or around December 18,

2018. If the right to terminate the Accelerated Book-Build Agreement (as defined herein) (see “—*Placement of Offered Shares*” in “Offering and Sale” beginning on page 147) is exercised, the Offering will lapse and any previously purported allocation and purchase of Offered Shares will be deemed not to have been made.

**Form of Offered Shares**

The Offered Shares will be issued as uncertificated securities (*Wertrechte*) within the meaning of article 973c of the CO and will be established as intermediated securities (*Bucheffekten*) within the meaning of the Swiss Federal Intermediated Securities Act of October 3, 2008, as amended (the “**FISA**”; *Bucheffektengesetz*).

The Offered Shares will be registered in the main register (*Hauptregister*) maintained by SIS and credited to the securities account of each purchaser, and thus will become intermediated securities (*Bucheffekten*) within the meaning of the FISA.

**Offering, Selling and Transfer Restrictions**

The Shares are subject to certain offering, selling and transfer restrictions as described in “*Notice to Prospective Investors*”, “—*Transfer of Shares and transfer restrictions*” in “*Description of the Company’s Capital Structure and Shares*”, and “*Selling and Transfer Restrictions*” beginning on pages iv, 138 and 151, respectively.

**Global Coordinator and Bookrunner**

UBS AG

**Co-Manager**

Mirabaud Securities Limited

**Listing Agent**

Homburger AG

**Law/Jurisdiction**

Swiss law/Zurich, Switzerland

**SIX Ticker Symbol**

SANN

**Swiss Security Number (*Valorennummer*)**

2714864

**International Security Identification Number (ISIN)**

CH 0027148649

**Notification/Amendments or Changes**

Any notices containing or announcing amendments or changes to the terms of the Offering or to this Offering Memorandum will be announced through the electronic media. Notices required under the Listing Rules will be published in electronic form on the website of the SIX Swiss Exchange (currently: [https://www.six-group.com/exchanges/news/official\\_notices/search\\_en.html](https://www.six-group.com/exchanges/news/official_notices/search_en.html)). Changes so notified will be deemed to constitute an amendment or supplement of this Offering Memorandum.

The Offer Price and the final number of Offered Shares sold in the Offering will be published via electronic media, by press release and in a pricing supplement to this Offering Memorandum on or around December 14, 2018.

### III. SUMMARY OF FINANCIAL INFORMATION

Unless otherwise stated, the summary historical financial information presented below as of and for the years ended December 31, 2017, 2016, and 2015 has been extracted or derived from the Audited Consolidated Financial Statements, which are included elsewhere in this Offering Memorandum. Unless otherwise stated, the summary historical financial information presented below as of and for the nine months ended September 30, 2018, has been extracted or derived from the Unaudited Interim Condensed Consolidated Financial Statements, which are included elsewhere in this Offering Memorandum.

The summary historical financial information presented below should be read in conjunction with, and is qualified in its entirety by reference to, the Audited Consolidated Financial Statements and the Unaudited Interim Condensed Consolidated Financial Statements.

The summary historical financial information presented below should also be read together with “*Management’s Discussion and Analysis of Financial Condition and Results of Operations*” beginning on page 43.

#### A. Summary financial information from the consolidated statement of comprehensive income

in CHF thousands	For the financial year ended December 31,			For the nine months ended September 30,	
	2015	2016	2017	2017	2018
	<i>(audited)</i>			<i>(unaudited)</i>	
<b>Net sales</b> .....	<b>4,321</b>	<b>19,033</b>	<b>22,943</b>	<b>16,347</b>	<b>23,634</b>
Cost of goods sold .....	(1,371)	(3,883)	(4,104)	(3,028)	(3,606)
<i>Of which amortization intangible asset</i> .....	<i>(1,013)</i>	<i>(3,039)</i>	<i>(3,039)</i>	<i>(2,279)</i>	<i>(2,279)</i>
Other operating income .....	188	361	270	243	1
Development .....	16,651	(17,675)	(26,561)	(18,168)	(27,098)
<i>Of which development expenses</i> .....	<i>(10,453)</i>	<i>(17,675)</i>	<i>(26,561)</i>	<i>(18,168)</i>	<i>(27,098)</i>
<i>Of which reversal impairment on intangible assets and inventory</i> .....	<i>27,104<sup>(1)</sup></i>	<i>0</i>	<i>0</i>	<i>0</i>	<i>0</i>
Marketing and sales .....	(8,356)	(21,051)	(28,522)	(19,909)	(18,637)
General and administrative .....	(8,244)	(9,805)	(14,416)	(9,573)	(11,292)
Other operating expenses.....	(16)	(107)	(64)	(68)	(169)
<b>Operating expenses</b> .....	<b>35</b>	<b>(48,638)</b>	<b>(69,563)</b>	<b>(47,718)</b>	<b>(57,196)</b>
<b>Operating result</b> .....	<b>3,173</b>	<b>(33,127)</b>	<b>(50,454)</b>	<b>(34,156)</b>	<b>(37,167)</b>
Financial income .....	416	928	4,134	3,533	2,856
Financial expenses.....	(655)	(995)	(4,955)	(3,605)	(5,232)
<b>Result before taxes</b> .....	<b>2,934</b>	<b>(33,194)</b>	<b>(51,275)</b>	<b>(34,228)</b>	<b>(39,543)</b>
Income taxes.....	3,015	(2,221)	(257)	895	(380)
<b>Net result</b> .....	<b>5,949</b>	<b>(35,415)</b>	<b>(51,532)</b>	<b>(33,333)</b>	<b>(39,923)</b>
Items never to be reclassified to net income in subsequent periods					
<i>Actuarial gains (losses) on defined benefit plans</i> .....	<i>(1,671)</i>	<i>(1,776)</i>	<i>(171)</i>	<i>137</i>	<i>1,936</i>
Items to be reclassified to net income in subsequent periods					
<i>Currency translation differences</i> .....	<i>(16)</i>	<i>(18)</i>	<i>82</i>	<i>60</i>	<i>(52)</i>
<b>Other comprehensive result</b> .....	<b>(1,687)</b>	<b>(1,794)</b>	<b>(89)</b>	<b>197</b>	<b>1,884</b>
<b>Total comprehensive result</b> .....	<b>4,262</b>	<b>(37,209)</b>	<b>(51,621)</b>	<b>(33,136)</b>	<b>(38,039)</b>

(1) As a result of receiving marketing authorization in the EU for Raxone® for the treatment of LHON in September 2015, an impairment of the Company’s main intangible asset “Raxone” in 2012 in the amount of CHF 26.2 million was reversed.

## B. Summary financial information from the balance sheet

in CHF thousands	As of December 31,		2017	As of September 30, 2018
	2015	2016		
	<i>(audited)</i>			<i>(unaudited)</i>
Tangible assets .....	398	517	2,157	2,344
Intangible assets .....	29,559	26,549	23,560	27,443
Financial assets long-term .....	190	270	713	775
Restricted cash long-term .....	0	0	4,500	1,500
Deferred tax assets .....	3,061	1,106	1,242	1,245
<b>Non-current assets.....</b>	<b>33,208</b>	<b>28,442</b>	<b>32,172</b>	<b>33,307</b>
Prepaid expenses and accrued income .....	1,513	583	853	804
Inventories.....	3,441	7,676	10,147	9,319
Trade and other receivables.....	2,131	4,276	5,402	6,953
Financial assets short-term.....	0	0	13,011	5,735
Restricted cash short-term.....	0	0	3,000	3,000
Cash and cash equivalents.....	76,859	49,815	45,195	19,654
<b>Current assets.....</b>	<b>83,944</b>	<b>62,350</b>	<b>77,608</b>	<b>45,465</b>
<b>TOTAL ASSETS.....</b>	<b>117,152</b>	<b>90,792</b>	<b>109,780</b>	<b>78,772</b>
Share capital.....	6,263	6,280	6,289	6,528
Capital reserves and share premium.....	377,031	382,322	392,002	403,159
Retained earnings.....	(273,133)	(308,549)	(360,081)	(400,004)
Employee benefit reserve .....	(2,958)	(4,734)	(4,905)	(2,969)
Treasury shares .....	(177)	(172)	(335)	(1,145)
Other components of equity .....	(779)	(796)	(714)	(766)
<b>Total equity.....</b>	<b>106,247</b>	<b>74,351</b>	<b>32,256</b>	<b>4,803</b>
Convertible bonds .....	0	0	53,111	54,193
Derivate financial instruments .....	0	0	2,792	1,069
Pension liabilities .....	3,957	6,183	8,375	6,879
<b>Total non-current liabilities.....</b>	<b>3,957</b>	<b>6,183</b>	<b>64,278</b>	<b>62,141</b>
Trade and other payables .....	3,666	4,458	4,734	3,463
Accrued expenses.....	3,282	5,800	8,512	8,365
<b>Total current liabilities .....</b>	<b>6,948</b>	<b>10,258</b>	<b>13,246</b>	<b>11,828</b>
<b>TOTAL LIABILITIES .....</b>	<b>10,905</b>	<b>16,441</b>	<b>77,524</b>	<b>73,969</b>
<b>TOTAL EQUITY AND LIABILITIES .....</b>	<b>117,152</b>	<b>90,792</b>	<b>109,780</b>	<b>78,772</b>

### C. Summary financial information from the cash flow statement

in CHF thousands	For the financial year ended December 31,			For the nine months ended September 30,	
	2015	2016	2017	2017	2018
	<i>(audited)</i>			<i>(unaudited)</i>	
<b>Result before taxes .....</b>	<b>2,934</b>	<b>(33,194)</b>	<b>(51,275)</b>	<b>(34,228)</b>	<b>(39,543)</b>
Depreciation of tangible assets .....	85	168	257	175	455
Reversal of impairment on intangible assets .....	(26,157)	0	0	–	–
Amortization of intangible assets .....	1,037	3,096	3,125	2,338	2,360
Expenses for equity rights plans .....	2,040	4,683	9,687	5,518	5,241
Change in fair value of derivatives .....	0	0	(2,540)	(2,344)	(1,723)
Change in fair value of financial assets short-term .....	0	0	(96)	(117)	249
Other non-cash items (Polyphor clinical material) .....	0	0	0	0	290
Change in pension liabilities .....	(394)	450	2,021	584	440
Taxes paid .....	(46)	(266)	(392)	(293)	(383)
Changes in net working capital .....	(2,119)	(2,131)	315	(2,046)	311
Total financial result .....	239	67	821	71	2,376
<i>Interest received</i> <sup>(2)</sup> .....	2	5	5	0	1
<i>Interest paid</i> .....	(11)	(15)	(1,561)	(1,541)	(3,033)
<b>Cash flow from operating activities .....</b>	<b>(22,390)</b>	<b>(27,137)</b>	<b>(39,633)</b>	<b>(31,883)</b>	<b>(32,959)</b>
Investments in tangible assets .....	(350)	(289)	(1,261)	(439)	(1,271)
Investments in intangible assets .....	(165)	(86)	(136)	(104)	(33)
Investments in other financial assets short-term .....	0	0	(12,915)	(12,915)	0
Disposal of other financial assets short-term .....	0	0	0	0	7,027
Investments in other financial assets long-term .....	(104)	(84)	(427)	(426)	(70)
Change in restricted cash .....	0	0	(7,500)	(7,500)	3,000
<b>Cash flow from investing activities .....</b>	<b>(619)</b>	<b>(459)</b>	<b>(22,239)</b>	<b>(21,384)</b>	<b>8,653</b>
Capital increases from options exercised .....	2,127	385	34	0	0
Proceeds from options exercised .....	0	0	0	21	0
Proceeds from sale of treasury shares .....	0	418	9,372	7,437	1,894
Purchase of treasury shares .....	0	(172)	(9,567)	(7,626)	(3,049)
Proceeds from convertible bonds .....	0	0	57,269	57,269	0
Capital increase private placement .....	54,870	0	0	0	0
Capital increase .....	27,576	0	0	0	0
Cost of issuance of share capital .....	(1,943)	0	0	0	0
<b>Cash flow from financing activities .....</b>	<b>82,630</b>	<b>631</b>	<b>57,108</b>	<b>57,101</b>	<b>(1,155)</b>
Effects of exchange rate changes on cash and cash equivalents .....	(197)	(79)	144	94	(80)
<b>Net increase (decrease) in cash and cash equivalents .....</b>	<b>59,424</b>	<b>(27,044)</b>	<b>(4,620)</b>	<b>3,928</b>	<b>(25,541)</b>
Cash and cash equivalents at January 1 .....	17,435	76,859	49,815	49,815	45,195
<b>Cash and cash equivalents at December 31 or September 30, respectively .....</b>	<b>76,859</b>	<b>49,815</b>	<b>45,195</b>	<b>53,743</b>	<b>19,654</b>

## IV. RISK FACTORS

*The Offering and any investment in the Shares are subject to a number of risks. Accordingly, prospective investors should carefully consider the risks and uncertainties described below, together with all other information contained in this Offering Memorandum, prior to making an investment decision.*

*The risks and uncertainties described below represent those we consider to be material as of the date of this Offering Memorandum. However, these risks and uncertainties are not the only ones we are facing. Additional risks and uncertainties not presently known to us, or that we currently consider not to be significant, could also materially and adversely affect our business, results of operations, financial condition or prospects. If any or a combination of these risks actually occurs, our business, results of operations, financial condition and/or prospects could be materially and adversely affected. In such case or cases, the price of the Shares could decline and prospective investors may lose all or part of their investment. This Offering Memorandum contains forward-looking statements that involve risks and uncertainties. The actual results could differ materially from those anticipated in such forward-looking statements as a result of certain factors, including the risks we face that are described below or elsewhere in this Offering Memorandum. The selected sequence of the risk factors mentioned below represents neither a statement about the probability of the risks' realization nor an assessment of the extent of the economic effects or the importance of the risks.*

*Investment decisions should not be made solely on the basis of the risk warnings set out in the Offering Memorandum since such information cannot serve as a substitute for individual advice and information that is tailored to the requirements, objectives, experience, knowledge and circumstances of each prospective investor individually. Therefore, before entering into any transaction, each prospective investor should consult with its own legal, regulatory, tax, financial and accounting advisors to the extent it considers necessary in order to determine whether an investment in the Shares is a fit, proper and suitable investment for it with a view to its financial situation, its constitutional documents, its internal policies and guidelines, the laws and regulations applicable to it and the impact an investment in the Shares will have on its overall investment portfolio. Only prospective investors who are fully aware of the risks associated with an investment in the Shares and who are financially able to bear any losses that may arise in connection therewith should consider engaging in any transactions in Shares.*

*Capitalized terms used but not defined herein have the meanings ascribed to them elsewhere in this Offering Memorandum.*

### A. Risks related to our business and financial situation

#### 1. Risks related to our financial position, capital needs and transactions

*Our ability to continue as a going concern depends on the successful completion of this Offering with gross proceeds of approximately CHF 50 million or, if we are unable to raise the targeted proceeds in this Offering, our ability to obtain such funding by way of another equity and/or debt financing in the immediate short term. A material uncertainty exists as to whether the Company's current funding is sufficient to support its going concern for another twelve months. Even if we are able to raise the targeted proceeds in this Offering or otherwise, we will continue to depend significantly on external equity and debt financing. We may not be able to obtain future financing or only obtain it on terms that significantly dilute the Company's shareholders and/or restrict our flexibility to operate.*

As at September 30, 2018, we had cash and cash equivalents of CHF 19.7 million, excluding restricted cash we placed in escrow for interest payments during the first three years of the term of our CHF 60 million Senior Unsecured Convertible Bonds 2017-2022 (the “**Bonds**”, see “—CHF 60 million Senior Unsecured Convertible Bonds 2017-2022” in “The Company and its Business” beginning on page 88 for more information). Sales in the first nine months of 2018 were CHF 23.6 million and we incurred a net loss for that period of CHF 39.9 million. Our operations have used substantial amounts of cash since our inception and we continue to require significant amounts of cash for operating our business and to satisfy our obligations.

We also expect our expenses to increase further in connection with our ongoing development activities as well as the ramping up of our commercialization activities relating to Raxone®.

We target gross proceeds from this Offering of approximately CHF 50 million, of which we intend to use the equivalent of USD 20.0 million to make the upfront payment for the option to obtain an exclusive sublicense from Idorsia in relation to vamorolone for the treatment of Duchenne Muscular Dystrophy (“DMD”) (see “*The Company and its Business—License and collaboration agreements*” beginning on page 84 for more information). We intend to complete the Offering as long as the net proceeds exceed the CHF equivalent of USD 20 million. If we are unable to raise the targeted proceeds of CHF 50 million in this Offering, we will need to raise additional funds by way of another equity and/or debt financing in the immediate short term in order to continue our operations as planned. Without such funds, there will be material uncertainty as to whether we will be able to continue as a going concern for another twelve months. The lower the proceeds that we are able to raise in this Offering, the more funds we will be required to raise by way of another financing in the immediate short term and the greater will be the risk that we will not be able to do so.

Pursuant to note 2 to the Unaudited Interim Condensed Consolidated Financial Statements, a material uncertainty exists as to whether the Company’s current funding is sufficient to support its going concern for another twelve months. The inclusion of a going concern qualification or emphasis of matter paragraph in any audit opinion related to our financial statements in the future may materially and adversely affect our trading and our ability to raise new capital that is needed to fund our operations.

Even if we are able to raise the targeted proceeds in this Offering, we will continue to depend significantly on external equity and debt financing, in addition to cash flows we generate from ongoing product sales and potential milestone payments. Such financing may not be available to us on acceptable terms, or at all, in particular in the short term. Also, the Bonds prohibit us from issuing any secured marketable debt instruments or incurring any secured financial debt (including bank debt) exceeding CHF 10.0 million in the aggregate (subject to exceptions) unless the Bonds are secured equally and rateably, or the Paying and Conversion Agent under the Bonds consents, which could adversely impact our ability to raise additional debt financing. If we fail to obtain additional funds on acceptable terms when needed, we may have to delay, reduce or terminate our product development programs or the production and commercialization of Raxone®, we may not be able to meet the cash requirements for operating our business and making payments with respect to our financial obligations, including interest and principal payments on our Bonds, and we may be required to file for bankruptcy.

If we are able to raise additional equity or issue equity-linked instruments, the Company’s shareholders could be significantly diluted. If we incur additional debt, the terms of such debt may subject us to restrictive covenants or security obligations that limit our flexibility in conducting future business activities, such as incurring additional debt or acquiring or licensing intellectual property rights.

***We are an early stage pharmaceutical company and have only one marketed product, Raxone® for the treatment of Leber’s hereditary optic neuropathy (LHON), which constitutes a relatively small business opportunity. In addition, we have one late-stage product candidate, Raxone® for the treatment of Duchenne Muscular Dystrophy (DMD), for which our recent applications for market authorization in the EU have been unsuccessful and for which we have not received marketing authorization for any country, as well as two early stage product candidates. We have incurred significant losses since our inception and expect to incur substantial losses and negative operating cash flows for the foreseeable future and may never achieve or maintain profitability.***

We are an early stage pharmaceutical company. We currently have no products approved for commercial sale other than Raxone®, for which we received marketing authorization in the European Union (the European Union or the European Economic Area, as applicable, the “EU”) in September 2015 for the treatment of Leber’s hereditary optic neuropathy (“LHON”), whose market is, however, small. We filed a marketing authorization application (“MAA”) with the European Medicines Agency (“EMA”) with respect to Raxone® for the treatment of DMD in certain patients with declining respiratory function who are currently not

taking steroids, which is an indication with respect to which we believe the potential market may be substantially larger than that for the treatment of LHON due to the larger number of patients affected by this condition. However, in September 2017, the EMA's Committee for Medicinal Products for Human Use ("CHMP") issued a negative opinion on our MAA, and in January 2018, the CHMP maintained such negative opinion following a re-examination procedure that we requested and despite an updated proposal for post-authorization measures and a clarification of the wording of the indication that we had proposed. See "Our lead product candidate: Raxone® in DMD—Market exclusivity and regulatory status" in "The Company and its Business" beginning on page 76 for more information.

We have incurred consistent cash-outflow and significant losses since our inception, including a net loss of CHF 21.2 million in 2015 (excluding the one-time effect of a reversal of an impairment), of CHF 35.4 million in 2016, of CHF 51.5 million in 2017 and of CHF 39.9 million in the nine months ended September 30, 2018. We expect to continue to incur significant operating losses for the foreseeable future, as we continue our development and commercialization efforts and make investments. We expect our expenses to increase substantially over the coming years, primarily due to higher operating expenses in connection with our ongoing development activities as well as the ramping up of our commercialization activities relating to Raxone®. To become and remain profitable, we must successfully complete the development of our product candidates, obtain marketing authorizations and pricing and reimbursement approvals for them (whereby references in this Offering Memorandum to our "product candidates" include in-licensed product candidates such as POL6014 and product candidates for which we have acquired an option to in-license such as vamorolone, unless stated or the context requires otherwise), expand our product pipeline, maintain and manage our manufacturing arrangements with third parties, maintain and build up an effective internal sales and marketing organization, establish and maintain sales and marketing arrangements with third parties and raise sufficient funds to finance our activities. We may never succeed in these activities, and even if we do, we may never generate sales that are significant enough to achieve profitability.

***Our marketed product, Raxone® in LHON, will not allow us to become profitable. Our future profitability, if any, will depend on us being able to obtain marketing authorization and, thereafter, pricing and reimbursement approvals for our product candidates, in particular Raxone® in DMD, as well as potentially in other indications.***

In 2017, we generated net sales of CHF 22.9 million with Raxone® in LHON, our only marketed product. Even if we reach the peak sales potential of Raxone® in LHON, this product alone will not allow us to become profitable. If Raxone® in LHON remains our only marketed product, we will not be able to become profitable and may eventually have to shut down our operations.

Our future success and profitability (if any) will depend on our ability to obtain marketing authorization and, thereafter, pricing and reimbursement approvals for Raxone® in DMD in the EU and in the United States of America (the "U.S."), as well as on other factors. We may never receive a marketing authorization for Raxone® in DMD (see preceding and next risk factor). Even if we eventually obtain such marketing authorization for the EU, we may not receive it on terms acceptable to us, or our product may not be commercially viable. Moreover, pricing and reimbursement decisions in the EU remain a competence of each Member State and therefore may vary significantly from one country to another. For a summary of risks related to pricing and reimbursement see the risk factor "The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain coverage and adequate reimbursement for our marketed product or any product for which we receive marketing authorization in the future and price controls could limit our ability to market those products and decrease our ability to generate sales."

We have unsuccessfully applied for marketing authorization for Raxone® in DMD patients with declining respiratory function who are currently not taking steroids, and have not yet sought marketing authorization for Raxone® in DMD patients who are receiving steroids, and our phase III clinical trial ("SIDEROS") for Raxone® in this subgroup of DMD patients is still ongoing and its outcome is uncertain. We have not filed a New Drug Application ("NDA") for Raxone® in DMD in the U.S., and there is considerable uncertainty around whether the U.S. Food & Drug Administration (the "FDA") would accept an NDA filing based on

the limited data from our phase III clinical trial of Raxone<sup>®</sup> in certain DMD patients with declining respiratory function who were not receiving steroids (“**DELOS**”). Even if the FDA accepts an NDA filing on this basis and grants the respective marketing authorization, a subsequent negative outcome of the SIDEROS trial could potentially jeopardize the maintenance of such marketing authorization.

If we are unsuccessful or significantly delayed in obtaining marketing authorization for Raxone<sup>®</sup> in DMD or its subsequent commercialization, or in the further commercialization of Raxone<sup>®</sup> in LHON, we would have to rely on the further development of vamorolone in DMD, for which we have acquired an option to in-license, our early stage pipeline that comprises omigapil in congenital muscular dystrophy (“**CMD**”) (currently in phase I clinical trial) and our recently in-licensed compound POL6014 in cystic fibrosis (“**CF**”) (currently in phase I clinical trial). Given the uncertainties around the development and commercialization of pharmaceuticals, we may not be able to develop and commercialize any such product candidates in a timely manner or at all.

***We may never receive a marketing authorization for Raxone<sup>®</sup> in DMD.***

The CHMP has issued a negative opinion on our MAA for Raxone<sup>®</sup> in certain DMD patients even after re-examination (see “*Our lead product candidate: Raxone<sup>®</sup> in DMD—Market exclusivity and regulatory status*” in “*The Company and its Business*” beginning on page 76). We may fail to collect further evidence to strengthen the clinical data package for Raxone in preparation of a refiling of an MAA with the EMA, and even if we are able to collect such additional data, we may not be able to make an improved case and there is a material risk that we cannot refile an MAA with the EMA. Even if we refile an MAA with the EMA, the CHMP may consider the additional data submitted by us not to be compelling enough to support a positive opinion by it and may issue a negative opinion on such MAA. Also, regulators elsewhere, in particular the FDA, if and when we file an NDA for Raxone<sup>®</sup> in DMD in the U.S. (see risk factor “*Our marketed product, Raxone<sup>®</sup> in LHON, will not allow us to become profitable. Our future profitability, if any, will depend on us being able to obtain marketing authorization and, thereafter, pricing and reimbursement approvals for our product candidates, in particular Raxone<sup>®</sup> in DMD, as well as potentially in other indications.*”), may be more reluctant to grant us marketing authorization for Raxone<sup>®</sup> in DMD given the negative opinion by the CHMP. If we do not receive a marketing authorization for Raxone<sup>®</sup> in DMD, we might have to abandon our development activities with regard to Raxone<sup>®</sup> in indications other than LHON.

***News on our development and commercialization efforts that we expect to receive during the coming months and in the longer term may have a significant and potentially adverse effect on the value of the Group and, as a consequence, the market price of the Shares.***

The value of the Group strongly depends on the results of our clinical trials and on the decisions by regulatory authorities. We expect to receive material new information on such matters in the coming months and in the longer term. In particular, depending on the outcome of discussions currently held with clinical experts and regulators, we may or may not be in a position to further develop omigapil in CMD. Moreover, we currently expect top line data of our phase III clinical trial of Raxone<sup>®</sup> in certain DMD patients with declining respiratory function who are receiving steroids (SIDEROS) in 2020. Such news or its delay may have a significant adverse effect on the value of the Group and adversely affect its business and prospects. As a consequence, the market price of the Shares is expected to be volatile. Should any such news be unfavorable, the market price of the Shares may significantly decline and, potentially, not recover.

***We may not realize the benefits of our recent in-licensing of POL6014 from Polyphor, of our recent option to in-license vamorolone from Idorsia, of any other product candidates or compounds that we may in-license or acquire, of any strategic alliances that we may form, joint ventures that we may create, or strategic transactions that we may enter into in the future.***

We have acquired, in-licensed or acquired an option to in-license all of our current product candidates, typically against payment of upfront consideration and milestone and royalty payments. In February 2018, we in-licensed the compound POL6014 from Polyphor against an initial consideration (paid in Shares) of CHF 6.5 million, and we agreed to cash payments of up to CHF 121 million contingent on future develop-

ment, regulatory and particularly sales milestones. In November 2018, we entered into the Option Agreement with Idorsia, under which we have acquired an option to obtain from Idorsia an exclusive sub-license to commercialize ReveraGen's vamorolone, a non-hormonal steroid modulator developed by ReveraGen. As consideration for the acquisition of the option for the exclusive sub-license relating to ReveraGen's vamorolone, we paid Idorsia an equity consideration of the 1,000,000 Idorsia Shares and have agreed to pay Idorsia a cash consideration in the amount of USD 20.0 million, which we intend to fund from the net proceeds from the issuance and sale of the Offered Shares. Under the Option Agreement, Idorsia will be entitled to receive a cash payment from us of USD 30.0 million upon exercise of the option and commercial milestone payments of up to USD 80 million in the DMD indication and four one-time sales milestone payments of up to USD 130 million in aggregate. Regulatory milestone payments payable by the Company to Idorsia for three additional indications amount to up to USD 205 million in aggregate. Upon commercialization of vamorolone, the Company has committed to pay to Idorsia tiered royalties ranging from a single-digit to low double-digit percentage on the annual net sales of vamorolone. Further to vamorolone, we are currently evaluating several additional potential in-licensing and acquisition opportunities in our three therapeutic areas. We may not be able to realize the benefit of our past or future acquisitions or in-licensing transactions, or they may turn out to have been made at too high a price. Likewise, any strategic alliances, joint ventures or strategic transactions that we may enter into in the future may fail to achieve the expected results and may divert capital resources and management time. It is unclear whether and when any product candidates may generate revenues for the Company.

## **2. Risks related to the development of our product candidates**

***Any setbacks impacting our only lead compound, idebenone (the active ingredient in Raxone®), may adversely affect our only marketed product and our only late-stage product candidate simultaneously.***

We rely on one lead compound, idebenone, for use in our marketed product, Raxone® in LHON and our late-stage product candidate, Raxone® in DMD. In aggregate, we rely on only three compounds (including idebenone) in our current development and commercialization efforts, and both our clinical development of omigapil in CMD and the development of POL6014 in CF are in early clinical stages (phase I clinical trial). Any adverse effects resulting from the use of idebenone in the human body, or any difficulty in the manufacture, or problem with the supply, of idebenone, or any measures taken by regulators in relation to idebenone, could adversely affect our marketed product and our late-stage product candidate simultaneously.

***Our product candidates must prove their efficacy and safety in rigorous clinical testing. Drug development involves a lengthy and expensive process, with an uncertain outcome. Failure may occur at any stage of clinical development.***

Before we may seek marketing authorization for any product candidate, we must conduct extensive clinical trials to demonstrate its safety and efficacy in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to its outcome. A failure of one or more clinical trials can occur at any stage of testing. Promising results in preclinical studies of a product candidate may not be predictive of similar results in humans during clinical trials, and successful results from early clinical trials may not be replicated in later and larger clinical trials or in clinical trials for different indications or different patient populations. For example, the results of our phase III clinical trial (DELOS) of Raxone® in certain DMD patients are not predictive of the results of our ongoing phase III clinical trial (SIDEROS) of Raxone® in a different DMD patient population; and the full results of our phase I clinical trial (CALLISTO) of omigapil in CMD, if they confirm the results of the top line data already available, are unrelated to the potential outcome of any phase II efficacy trial of omigapil in CMD that we might decide to start in the future.

***The conduct of clinical trials may be prevented, delayed, or even futile, and delays in the commencement, enrollment or completion of clinical trials for any of our product candidates could result in increased costs, or prevent us from commercializing our product candidates on a timely basis, or at all.***

Before a clinical trial may begin, we or our partners must obtain approval from the competent regulatory authority and/or the competent ethics committee. We or our partners may not obtain authorization for further testing of our product candidates. Clinical trials of our product candidates may not be conducted as planned, and commencement, enrollment or completion may not occur on our planned schedule, if at all, for many reasons, which could result in increased costs and could negatively affect our or our partners' ability to complete the clinical trial. We have experienced delays in clinical trials and cost overruns in the past and may do so again in the future. If we or our partners are not able to successfully design, operate, complete and correctly evaluate the results of the clinical trials for our product candidates, we will not be able to seek marketing authorization or commercialize them.

***If we or our partners experience delays or difficulties in the enrollment of patients in clinical trials, the conduct and completion of clinical trials may be delayed or prevented. Also, the availability of idebenone from inexpensive sources may adversely affect patient enrollment or the results of our clinical trials.***

Initiation and successful and timely completion of clinical trials requires us to enroll a sufficient number of eligible patients in these trials. Given our focus on orphan drugs, our clinical trials look to enroll patients with characteristics that are found in a small number of patients and are likely to compete with other clinical trials for product candidates targeting treatment of patients with the same characteristics. As the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in these clinical trial sites. As idebenone can be purchased over the internet and via other inexpensive sources, patients may be reluctant to enroll in our clinical trials where they do not know whether they will receive idebenone or a placebo. Also, the parallel use of idebenone by patients in the placebo arm of a trial may adversely affect the results of our clinical trials.

Delays in patient enrollment may result in increased costs or may affect the timing or outcome of our clinical trials, which could prevent their completion. We are, for example, in the process of enrolling patients for a phase III clinical trial (SIDEROS) for Raxone® in certain DMD patients. Enrollment is still ongoing and has been slower than anticipated. Based on our current estimates, we expect top line data of the SIDEROS trial to become available in 2020. Should there be any further delays in patient enrollment or if we are unable to recruit enough patients, the SIDEROS trial (and, consequently, the availability of any data) could be significantly delayed or even prevented.

***We may not be successful in our efforts to build up our pipeline of product candidates or to spend our limited resources on the most promising product candidates.***

We may not be able to develop our existing product candidates or identify and develop further product candidates that are safe and effective despite spending substantial technical, financial, and personnel resources thereon. Because we have limited resources, we may forgo or delay pursuit of opportunities with certain product candidates or indications that later prove to have a greater potential than the product candidates or indications on which we have chosen to focus. Even if we are successful in continuing to build our pipeline, the product candidates that we identify may not be suitable for clinical development, including as a result of being shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing authorization and achieve market acceptance.

***We rely and will in the future rely on third parties to conduct clinical trials for our product candidates, and if they do not properly and successfully perform their obligations, we may not be able to successfully complete the respective development of our product candidates.***

We rely on Clinical Research Organizations (“CROs”) and other third parties to assist in managing, monitoring and otherwise carrying out clinical trials for our product candidates. Together with the salaries paid to our employees in the product development department, the fees and expenses of these CROs make up

most of our development expenses. We compete with many other companies for the resources of these third parties. These third parties generally may terminate their engagements with us at any time.

If the quality or accuracy of the data that these third parties obtain is compromised due to their failure to adhere to clinical trial protocols or to regulatory requirements, or if they otherwise fail to comply with clinical trial protocols or meet expected deadlines, the clinical trials of our product candidates may not meet regulatory requirements. In one case, we had to terminate our relationship with a CRO for cause in 2016 and had to engage another CRO to complete the clinical trial conducted by the former CRO. If clinical trials do not meet regulatory requirements or if these third parties need to be replaced for any reason, the development of our product candidates may be delayed or suspended, may be more expensive than planned or may ultimately fail.

Although we rely extensively on third parties to conduct our product development work, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with its general investigational plan, protocol, legal and regulatory requirements and scientific standards. We may incur financial liabilities or suffer negative regulatory consequences as a result of any shortcoming in meeting such responsibilities irrespective of whether we have delegated such responsibility to a CRO or other third party.

***We may not be successful in maintaining existing or establishing and maintaining additional collaborations.***

We may not be successful in implementing our agreement with Idorsia relating to the acquisition of an option for the exclusive sub-license relating to ReveraGen's dissociative steroid vamorolone.

We conducted the phase I clinical trial (CALLISTO) of our product candidate omigapil in CMD in collaboration with the National Institute of Neurological Disease and Strokes (the "NINDS"), an institute within the National Institutes of Health (the "NIH") in the U.S. (see "*—Omigapil as phase I product candidate in CMD—Clinical development status*" in "*The Company and its Business*" beginning on page 82 for more information). This existing collaboration is, and any future collaborations or partnerships may be, important to our business. Generally, such collaborations allow us to share the development costs with our collaboration partners, thereby significantly reducing our own costs, and to utilize the expertise and know-how of our development partners. If a collaboration partner collaborates with us after assessing the viability of a product candidate, we also consider this a validation of our own development effort.

We may not be able to maintain our current or any future collaborations or partnerships, including for reasons beyond our control. In the event of termination of a collaboration, we may be unable to progress the relevant product candidate on our own or may be unable to successfully find a new partner with which to do so on terms favorable to us or at all. Also, any termination of a collaboration by our partner could make it difficult for us to attract new strategic partners or adversely affect how we are perceived in scientific and financial communities.

We will face significant competition in seeking partners for future product development collaborations. In order for us to successfully partner our product candidates, potential partners must view the respective product candidate as attractive, also in light of the terms that we are seeking. Even if we successfully establish new collaborations, their terms may not be favorable to us.

If we fail to establish or maintain a collaboration related to a particular product candidate, we will bear all of the related development cost and risk and may be unable to develop that product candidate on our own for lack of resources or other reasons.

***If serious adverse events or undesirable or unacceptable side effects are identified during the development of any of our product candidates or after commercialization of any product or any future products,***

*we may need to abandon the development of the product candidates or withdraw the product from the market.*

If any of our product candidates cause undesirable or unacceptable side effects in clinical trials or have characteristics that are unexpected, we may decide or be required to interrupt, delay or abandon the relevant product candidate's development or may choose to limit its development to more narrow uses or patient subpopulations in which such side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Serious procedure- or treatment-related side effects may occur at any stage of product development and even after commercialization. Any such side effects may cause us to abandon or limit the development of the relevant product candidate or we may decide or be required to withdraw the relevant product from the market, which may result in a sudden and sharp drop of our net sales and/or significant impairment charges. These risks are amplified by the fact that we rely on one lead compound, idebenone, for use in our only marketed product, Raxone® in LHON and our only late-stage product candidate, Raxone® in DMD.

### **3. Risks related to marketing approval of our product candidates and legal compliance matters**

*Following clinical development, our product candidates will require marketing authorization. If we are not able to obtain marketing authorization for a particular product candidate in a timely manner, on terms acceptable to us or at all, we will not be able to commercialize it, and our ability to generate sales will be materially impaired.*

Our product candidates require marketing authorizations from the FDA in the U.S., from the European Commission in the EU and from comparable regulatory authorities in other relevant jurisdictions (such as Swissmedic in Switzerland), prior to commercialization. In most jurisdictions, the process of obtaining marketing authorization for a product candidate is expensive and may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Failure to obtain marketing authorization for a product candidate will prevent us from commercializing the product candidate.

Our experience in filing and supporting the applications necessary to gain marketing authorization for a product candidate is limited and some of our marketing applications were not successful. So far, only two marketing authorizations for our product, Raxone® in LHON, have been granted: we received marketing authorization for our product from the European Commission, and our exclusive distributor received marketing authorization for it from the Israeli Ministry of Health. We also filed MAAs with the EMA and with Swissmedic for Raxone® in certain DMD patients, but failed to receive marketing authorization for the EU and, as a result, withdrew our MAA with Swissmedic. We have never filed an NDA with, or obtained marketing authorization from, the FDA in the U.S., which is a significant pharmaceutical market. We have started (but may fail) to build up our in-house capacity for purposes of obtaining marketing authorization for Raxone® in DMD in the U.S., and we continue to rely on external advisors to assist us with the marketing authorization process in the U.S.

Regulatory authorities have substantial discretion in the timing and substance of the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. For example, the CHMP, when it issued its negative opinion on our MAA for Raxone® in certain DMD patients in the EU, considered that the effect of Raxone® on patients' respiratory function observed in our phase III clinical trial of Raxone® in certain DMD patients with declining respiratory function who were not receiving steroids (DELOS) could be clinically relevant if it would be maintained over several years, rather than the 52 weeks observed in the DELOS trial. The CHMP also expressed concerns about the way the study was conducted and analyzed. Moreover, there is considerable uncertainty around whether the FDA would accept an NDA filing based on the limited data from our DELOS trial, and it is possible that the FDA may not consider the SIDEROS trial, or any additional studies for Raxone® in DMD performed and completed that it may request, sufficient to approve any NDA for Raxone® in DMD that we may submit. If our clinical data are found insufficient, we may be forced to abandon an MAA for the EU or an NDA for the U.S. with respect to Raxone® in certain DMD patients.

Regulatory authorities may also narrow the uses or patient subpopulations for which the product is approved or require extensive warnings on the label, thereby limiting the potential market for or interest in the product.

If we experience delays in obtaining or fail to obtain marketing authorizations for any of our product candidates in any key jurisdiction, especially in the U.S. and the EU, their commercial prospects may be harmed or they may no longer be commercially viable. As a result, our ability to generate sales will be materially impaired.

***Fast track, breakthrough therapy and similar designations for some of our product candidates may not lead to a faster development or regulatory review or approval process, will not increase the likelihood of receiving marketing authorization and may be revoked.***

We have received fast track designation and rare pediatric disease designation from the FDA for Raxone<sup>®</sup> in DMD and for omigapil in CMD (see “—Our lead product candidate: Raxone<sup>®</sup> in DMD—Market exclusivity and regulatory status” in “The Company and its Business” beginning on page 76 and “—Omigapil as phase I product candidate in CMD—Market exclusivity and regulatory status” in “The Company and its Business” beginning on page 82, respectively, for more information). We may seek fast track or similar designations for POL6014 in CF and/or any future product candidates. Further, the UK’s Medicines and Healthcare Products Regulatory Agency (the “MHRA”) gave certain DMD patients access to Raxone<sup>®</sup> under the Early Access to Medicines Scheme (“EAMS”) following its designation as Promising Innovative Medicine (“PIM”) by the MHRA (see “—Our lead product candidate: Raxone<sup>®</sup> in DMD—Market exclusivity and regulatory status” in “The Company and its Business” beginning on page 74 for more information). We may seek, but may not necessarily receive designations comparable to breakthrough therapy designations or PIM in other jurisdictions and for other products or product candidates.

Regulatory authorities typically have broad discretion in granting fast track, break through therapy, PIM and similar designations and may rescind or revoke such designations. Even if such designation is granted, such designation is not predictive of future clinical trial results, does not necessarily (and in the case of certain designations will not) result in a faster development process, review or marketing approval compared to conventional approval procedures and does not increase the likelihood that a product candidate will receive marketing authorization. Many drugs that have received such designations have failed to obtain marketing authorization. If we fail to obtain any such designation for a product candidate that we think meets the criteria or any existing designations is revoked, further development of that product candidate and, ultimately, its commercialization could be materially adversely affected.

***Our marketed product Raxone<sup>®</sup> in LHON is, and any product candidate for which we may obtain marketing authorization will be, subject to extensive post-marketing regulatory requirements and could be subject to post-marketing restrictions, post-marketing studies or withdrawal from the market, and we may be subject to penalties if we or the third parties with which we collaborate fail to comply with regulatory requirements or experience unanticipated problems with that product.***

Our commercialization activities with respect to Raxone<sup>®</sup> in LHON (our marketed product) are, and any product candidates for which we may receive marketing authorization will be, subject to comprehensive regulation by regulatory authorities in each jurisdiction in which it is authorized. This regulation includes requirements regarding the testing, manufacture, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution of the relevant product. For example, for any marketed product we will need to submit safety and other post-marketing information and reports, ensure that our contract manufacturers observe current Good Manufacturing Practice (“cGMP”) requirements and comply with requirements regarding safety monitoring and pharmacovigilance.

Regulatory authorities may also impose requirements for expensive post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product. For example, under the European Commission’s marketing authorization that was granted for Raxone<sup>®</sup> in LHON “under exceptional circumstances” because the European Commission found that comprehensive efficacy and safety data cannot be obtained, we are required to conduct several post-authorization measures, which include an additional

phase IV clinical trial on the long-term effects and safety of Raxone® in LHON and a second comparative natural history study that we are currently conducting, as well as maintenance of a registry of LHON patients treated with Raxone® (see “—Market exclusivity, regulatory status and sales” in “The Company and its Business” beginning on page 73 for more information).

Also, if we refile an MAA for Raxone® in certain DMD patients with the EMA based on additional data in the future, even if the CHMP recommends that the European Commission grant marketing authorization, such marketing authorization would very likely be subject to the condition that we conduct a post-authorization safety study (“PASS”) and an externally controlled long-term open label study as post-authorization measures (see “—Our lead product candidate: Raxone® in DMD—Clinical development status” beginning on page 75 for more information). Any such requirements for Raxone® in DMD or for any other products for which we may receive marketing authorization in the future may adversely affect our profit and cash flow generated from the relevant products, and such additional clinical trials involve the risks associated with any clinical trials. For example, if our phase IV clinical trial of Raxone® in LHON does not establish the product’s long-term efficacy, this may impact its commercial success. Also, later discovery of previously unknown adverse effects or other problems with our products, manufacturers or manufacturing processes, or non-compliance with regulatory requirements may have serious consequences for us, including legal or regulatory actions such as warning letters, suspension of manufacturing, seizure of product, injunctions, withdrawal of the relevant product from the market and sanctions.

***Our relationships with customers and third-party payers and our general business operations are and will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm or diminished earnings, among other penalties.***

Healthcare providers and third-party payers play a primary role in the recommendation and prescription of Raxone® in LHON and any product candidates for which we may obtain marketing authorizations. The arrangements with healthcare professionals, third-party payers and customers that we or our distributors have entered or will enter into may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we or our distributors market, sell and distribute our products (for which we receive marketing authorization). Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. If authorities conclude that our or our distributors’ business practices do not comply with applicable laws and regulations, we or our employees or distributors may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government-funded healthcare programs such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, or the curtailment or restructuring of our operations.

***If we or our third-party contractors or employees fail to comply with environmental, health and safety laws, we could become subject to civil or criminal penalties, other remedial measures or incur costs that could harm our business.***

We are subject to a variety of environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of biological materials and hazardous materials and wastes. The operations of our third-party manufacturers and suppliers involve the use of hazardous and flammable materials, including chemicals and biological materials, and also produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials or wastes. In the event of such contamination or injury, we could be held liable for any resulting damages, and any liability could exceed our insurance coverage and our own resources. Non-compliance by us or our third-party contractors or employees with environmental, health and safety laws and regulations may result in substantial fines, civil or criminal penalties or other sanctions. In addition, we may incur substantial costs in order to comply with such laws and regulations.

#### 4. Risks related to the commercialization of our product candidates and marketing and sale of our products

*Our marketed product, Raxone<sup>®</sup> in LHON, and any of our product candidates (to the extent we receive marketing authorization for them) may fail to achieve the degree of market acceptance by physicians, patients, third-party payers and others in the medical community necessary for commercial success despite having received marketing authorization.*

Raxone<sup>®</sup> in LHON (our marketed product), and any product candidates, if any, for which we receive marketing authorization in the future, may fail to gain sufficient market acceptance by physicians, patients, third-party payers and others in the medical community despite having received marketing authorization. For example, other novel products may be preferred to our product. If any such products do not achieve an adequate level of acceptance, we may not generate significant product sales and we may not become profitable.

*Off-label and unlicensed uses of currently available forms of idebenone may adversely affect our sales of Raxone<sup>®</sup>.*

Physicians may prescribe available products containing idebenone (the active ingredient in Raxone<sup>®</sup>) for uses for which they are not approved, such as the treatment of LHON or DMD, if they view such products as a less expensive treatment or a better alternative to Raxone<sup>®</sup>. A considerable number of physicians in Europe, and to a lesser degree in the U.S. and other countries, have been prescribing or recommending products containing idebenone to their patients on an off-label basis. The off-label product is either acquired from internet sources or in countries where it is approved and marketed for a different indication. By way of example, and without any claim to completeness:

- Takeda's Mnesis<sup>®</sup>, 45mg tablets containing idebenone, is registered in Italy for the treatment of "cognitive-behavioral deficits resulting from cerebral pathologies whether from vascular or degenerative origin" and is used off-label and prescribed as an unlicensed medicine for the treatment of other (non-approved) indications in Italy and in certain other countries.
- Sweden's Medical Products Agency ("MPA") has granted several licenses to individual patients for the prescription and reimbursement of Mnesis<sup>®</sup> for the treatment of LHON. We have initiated a number of court proceedings to challenge these MPA decisions. The Swedish Supreme Administrative Court has not granted leave to appeal first instance decisions that we do not have standing to challenge the respective licenses. Consequently, several of these court proceedings have been dismissed. However, there are also court proceedings pending based on new case law supporting that we should have standing to challenge the respective licenses. While we believe, based on advice of our counsel, that refusing us standing to challenge the respective licenses is an erroneous application of law, there can be no assurance that the administrative courts will agree with our view.
- Pharmacies have been compounding idebenone. See risk factor "*Pharmacies have been compounding idebenone. Future compounding may adversely affect our sales of Raxone<sup>®</sup>.*"

Any off-label or unlicensed use of idebenone, especially from inexpensive sources, and any reimbursement for such use granted by third-party payers may reduce our potential sales of Raxone<sup>®</sup>.

*We have only started to develop our marketing and sales organization, have limited experience in marketing products and do not expect to have significant marketing synergies between our current marketed product and, if and when approved, our current product candidates. If we are unable to establish and expand our marketing and sales capabilities or enter into distribution agreements with third parties, we may not be able to generate product sales.*

We have only started to develop our own marketing, sales and distribution capabilities and have yet to commercialize Raxone<sup>®</sup> in LHON outside the EU. We have limited experience in marketing products in

Europe and have no experience in marketing products in the U.S. and elsewhere. We are marketing Raxone® in LHON in European countries through a small internal sales and marketing force that we have been building up since January 2015, through the third-party distributor Ewopharma in eleven countries in Eastern Europe and the Baltics and the third-party distributor Pharmathen in Greece and Cyprus, each on an exclusive basis. In the U.S., our team currently manages our patient advocacy interactions, prepares for market entry in the U.S. and is the source of our U.S. regulatory and medical affairs expertise, whereas commercialization will only be possible if we file an NDA with, and receive marketing authorization from, the FDA regarding Raxone® in DMD.

In connection with the further rollout of Raxone® in LHON in the EU, and as a result of the ramp up of our activities in the U.S., we will need to develop further in-house marketing, sales and distribution capabilities. All of these activities are associated with an increase in the headcount of our marketing and sales personnel and of the related overhead and higher overall fixed costs, and will also require significant management resources and time. At the same time, we may engage additional third-party distributors to perform marketing, sale and/or distribution services. Any income that we receive or may receive from our current or future third-party distributors will depend upon the efforts of such distributors, over which we may have little or no control. We may not be able to develop and expand in-house marketing, sales and distribution capabilities or establish or maintain relationships with third-party collaborators to successfully commercialize Raxone® in key markets and for particular indications, which would adversely impact our ability to generate product sales.

Raxone® in LHON and each of our product candidates (if approved) will have different prescriber bases: primarily ophthalmologists in the case of Raxone® in LHON, primarily neurologists in the case of Raxone® and vamorolone in DMD and omigapil on CMD, and primarily pulmonologists in the case of POL6014 in CF. As a result, we expect to have somewhat limited marketing synergies between our products and may have to build separate sales channels for each of our products, which is expensive and may result in our products suffering from low profit margins or a lack of profitability.

***We face substantial competition, which may result in others discovering, developing or commercializing competing products before or more successfully than we do, as well as reducing the price at which we are able to sell our products.***

The development and commercialization of new products is highly competitive. For instance, we believe that companies that are currently developing new products for the treatment of LHON (which may compete with our own product, Raxone®) may be granted marketing authorization during the next several years. Also, to our knowledge, two treatments for DMD developed by third parties that are not based on steroids have been approved to date, and there are a number of phase II clinical trials of drugs targeting muscle weakness in DMD. For an overview of the competitive landscape of our product and product candidates see “—Competition” in “*The Company and its Business*” beginning on page 90 and the references cited therein. The fact that our lead compound, idebenone (the active ingredient in Raxone®), does not enjoy composition of matter patent protection lowers entry barriers for competitors.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive or are better marketed than Raxone® in LHON or any product candidates for which we receive marketing authorization. Our competitors may obtain marketing authorizations for their products more rapidly than we do, which could result in them establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected by third-party payers seeking to encourage the use of products that are less expensive than ours.

***Should we or our distributors be found to have improperly promoted off-label uses, we may become subject to significant liability.***

Given that our marketed product, Raxone® in LHON, is also a product candidate for DMD, physicians may prescribe Raxone® to their patients in a manner that is inconsistent with our existing marketing authorizations in the EU and (via our exclusive distributor) in Israel or any future marketing authorizations. If we

cannot successfully manage the marketing of our products by restricting off-label promotion or if we or our current or future distributors promote our products beyond their approved indications, we could become subject to enforcement action for off-label promotion and significant liability.

***The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain coverage and adequate reimbursement for our marketed product or any product for which we receive marketing authorization in the future and price controls could limit our ability to market those products and decrease our ability to generate sales.***

The availability and extent of coverage and reimbursement by governmental and private third-party payers is essential for most patients to be able to afford expensive treatments. Sales of Raxone® in LHON (our marketed product) and any products for which we receive marketing authorization in the future will depend substantially on the extent to which the costs will be paid by third-party payers. Also, we rely on the efforts of our exclusive third-party distributor Ewopharma to obtain pricing and reimbursement approvals in eleven countries in Eastern Europe and the Baltics, as well as other third-party distributors, and may enter into similar arrangements with other third parties for other territories. We may have little or no control over the efforts of such third parties.

Seeking third party reimbursement is a time-consuming and expensive process, which typically requires us to provide scientific and clinical support and pharmaco-economic arguments for the use of the relevant product to each third-party payer separately. We may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, and third-party payers may nonetheless refuse reimbursement. Also, one third-party payer's decision to provide coverage for a product does not assure that other payers will also provide coverage, and pricing negotiations may continue after reimbursement has been obtained. The sales uptake of Raxone® in LHON in 2016 and 2017 was slower than originally expected due to the complex pricing and reimbursement processes in several EU markets and may continue to be slower than originally expected. As of the date of this Offering Memorandum, full reimbursement of Raxone® in LHON has been achieved for Germany, the Netherlands, Italy, Sweden, Scotland and six other jurisdictions. In several other jurisdictions, including France and England and Wales, Raxone® in LHON is currently covered by special reimbursement schemes. Third-party payers in several major EU countries have rejected our requests for pricing and reimbursement and we have been involved in legal proceedings in relation to such decisions. Even where reimbursement was approved or may be approved in the future, we had or may have to grant a significant discount on the list price and may have to reduce the price further in the future. Irrespective of the level of initial pricing, we expect the prices of our current and any future products to erode substantially during any market exclusivity period. We expect such price erosion to be accelerated after we have lost any such market exclusivity.

If reimbursement is not available or only to limited levels, we may not succeed in commercializing a product even if marketing authorization has been obtained. Even if coverage is provided, the approved reimbursement amount may not allow us to realize a sufficient return on our investment.

***Recently enacted and future healthcare reform legislation involves a high degree of uncertainty and may adversely affect our business.***

We operate in a highly regulated industry. New laws, regulations or judicial decisions, or new interpretations of existing laws, regulations or decisions, related to healthcare availability, the method of delivery or payment for healthcare products and services could adversely affect pricing, reimbursement, marketing or sales of our marketed product or any product candidates for which we may receive marketing authorization in the future. In the United States and other jurisdictions, there have been a number of legislative and regulatory changes, proposed changes and statements by the current President of the United States regarding the pharmaceutical industry and the healthcare system that could prevent or delay marketing authorization and pricing and reimbursement approvals of our product candidates or make them more expensive, or their terms less attractive, or restrict or regulate post-approval activities. In particular, we may face uncertainties as a result of U.S. federal legislative and administrative efforts to repeal, substantially modify or invalidate some or all of the provisions of the Patient Protection and Affordable Care Act as amended by the Health Care and Education Reconciliation Act (collectively, the "PPACA"). A repeal or replacement of the

PPACA, if it occurs, may adversely affect our business and financial results. All of these enacted or future measures may prevent us from generating sales, attain profitability, commercialize or market our products.

***Pharmacies have been compounding idebenone. Future compounding may adversely affect our sales of Raxone®.***

Compounding (also called pharmacy or magistral preparation) is a practice in which a licensed pharmacist prepares medicines in a pharmacy by combining, mixing, or altering pharmaceutical ingredients. Under certain conditions, the sale of compounded idebenone (the active ingredient of Raxone®) is legal. In the EU, such compounding exemption is based on Article 3 of the EU Directive 2001/83. We are aware of pharmacies in Germany and the Netherlands that advertised compounded idebenone on the internet for the treatment of LHON, DMD and other indications at considerably lower prices than we charge for Raxone® in LHON, sometimes making reference to the clinical trials of Raxone® that we have conducted.

Compounding of idebenone has also resulted in litigation: a pharmacist in Germany filled capsules with generic idebenone purchased from a third party and advertised their sale on his website. In August 2017, the Landgericht Hamburg prohibited the pharmacist's advertising and sale of idebenone capsules, holding among other things that the portioning of the active pharmaceutical ingredient and filling of capsules are not covered by the compounding privilege. The pharmacist has appealed this decision. In addition, we are aware of the case of an LHON patient in Austria whose third-party payer—a major third-party payer in Styria, Austria—decided to reimburse the costs of compounded idebenone, but not of Raxone®. The patient successfully challenged this decision in court and the third-party payer must reimburse the costs of Raxone®. Further, we are aware that a major third-party payer in Vienna denied a LHON patient the reimbursement of Raxone®, arguing that pharmacists' preparations are more cost efficient; this patient has initiated legal proceedings against the third-party payer.

Irrespective of the outcome of these cases, compounding of idebenone still continues and, consequently, reduces our sales of Raxone® in LHON and, eventually, if we receive marketing authorization, in DMD.

***Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of our marketed product or any product candidates for which we receive marketing authorization in the future.***

We face an inherent risk of product liability exposure related to our commercialization of Raxone® in LHON (our marketed product) and any product candidate for which we receive marketing authorization in the future, as well as to the use of our product candidates in humans in clinical trials. If we cannot successfully defend ourselves against claims that our products or product candidates caused injuries, we may incur substantial liabilities.

We currently have product liability insurance for Raxone® in LHON and insurance for human clinical trials covering all clinical trials conducted by us. However, our current product liability coverage may not be adequate in scope to protect us in the event of a successful product liability claim. Further, we may not be able to maintain our current insurance or obtain product liability insurance for any products for which we may receive marketing authorization in the future on reasonable terms and at acceptable cost, and our insurance may in any event provide insufficient coverage against potential liabilities. As a result, we may have to bear substantial uninsured losses.

***Our future profitability may be adversely affected if our estimates regarding the size of the market for our products and product candidates are inaccurate.***

We based our estimates regarding the size of the market for our products and product candidates on our experience and our evaluation of market conditions, using publicly available information. In formulating these estimates, we made certain assumptions, which have not been verified by third parties. If our assumptions are incorrect, there is a risk that our estimates could be wrong and our future profitability may be adversely affected. For example, we may not be able to realize the currently estimated peak sales potential of approximately USD 500 million of vamorolone in DMD due to a number of factors, including but not

limited to any of the assumptions made by us in estimating this number being inaccurate or not applicable or not materializing in the future as and when anticipated.

## 5. Risks related to market exclusivity rights and intellectual property

***Our business model relies on orphan drug exclusivity for our marketed product, Raxone<sup>®</sup> in LHON, and most of our current or future clinical product candidates. Orphan drug designation can be difficult to obtain and maintain, and it provides only limited protection from competition.***

It is our strategy to develop and commercialize product candidates in indications qualifying for orphan drug designation in order to obtain marketing exclusivity. For more information on orphan drug designation and its potential benefits see “—Market exclusivity and intellectual property” in “*The Company and its Business*” beginning on page 90. The market exclusivity period of an orphan drug designation is generally shorter than a patent protection period. In the U.S., for instance, such period is seven years, and in the EU, it is 10 years (reduced to six years if the relevant drug no longer meets the criteria or is sufficiently profitable) after receipt of marketing authorization. Also, orphan drug exclusivity may be lost if the applicable regulatory authority determines that the request for designation was materially defective, if the manufacturer is unable to assure sufficient quantity of the drug to meet patient needs, or for other reasons.

To date, we have obtained orphan drug designations (a) for our marketed product, Raxone<sup>®</sup> in LHON, in the EU (maximum duration until fall 2025), the U.S. and South Korea; (b) for our product candidate Raxone<sup>®</sup> in DMD, in the EU and the U.S.; (c) for our product candidate omigapil in CMD, in the EU and the U.S.; and (d) for POL6014 in alpha-1 antitrypsin deficiency (“AATD”), primary ciliary dyskinesia (“PCD”) and cystic fibrosis (“CF”) in the EU. We plan to seek orphan drug designation for POL6014 in CF in the U.S. as well, but may fail to obtain it. Vamorolone has received orphan drug designation in the U.S. and in the EU. We have not filed for orphan drug designation in all national and regional jurisdictions where such protection may be available; instead, we have sought such protections only with respect to jurisdictions that we currently anticipate being key to our business.

Obtaining an orphan drug designation can be difficult, and we may not be successful in obtaining or maintaining orphan drug designations for our marketed product or any of our product candidates. The procedure for obtaining orphan drug designation is an independent procedure in each jurisdiction, and applications might be denied in some jurisdictions, but granted in others. Further, orphan drug designation may be obtained for the same product in the same indication by several parties, and only the first such party to obtain marketing approval will receive marketing exclusivity for the relevant product in the relevant indication. Consequently, despite us having obtained an orphan drug designation for a product candidate in a particular indication, if a third party were to obtain orphan drug designation and marketing authorization and the correspondence market exclusivity for the same product in the same indication, we would be excluded from marketing such product in such indication during the applicable exclusivity period.

If we lose orphan drug designation or fail to maintain that designation for the duration of the applicable exclusivity period in relation to our marketed product or, after receipt of marketing authorization (if any), any of our product candidates, we may be unable to generate sufficient sales from such product or product candidate to become profitable.

***Our marketed product, Raxone<sup>®</sup> in LHON, is not patent protected and we may only be able to seek limited patent protection, if at all, for most of our product candidates. Even granted patents may not be enforceable, and we may be subject to ownership disputes over patents or other intellectual property.***

As the composition of matter patent for our lead compound, idebenone (the active ingredient in Raxone<sup>®</sup>) has expired, we can only seek method of use patent protection, as we have done for the use of idebenone to treat DMD. Typically, the protection derived from method of use patents is not as strong as the protection derived from composition of matter patents. Method of use patents do not prevent a third party from using, applying or manufacturing the same compound for other indications and may not prevent a third party from finding a way to circumvent the patent. For these reasons, a third party may be able to use idebenone in

different or comparable formulas, applications or indications. Further, method of use patents are, in general, more susceptible to invalidity attacks by third parties than composition of matter patents.

Raxone® in LHON (our marketed product) is not patent protected. Our method of use patents for the use of Raxone® in DMD are due to expire in March 2026 in the EU, Japan, and the U.S. Most composition of matter patents for omigapil, including those in the U.S. and the EU, with regard to which we have an exclusive license from Novartis, have expired. Our method of use patents for the use of omigapil in CMD in the U.S., the EU and other jurisdictions are due to expire in 2026 or 2027, as applicable. The composition of matter patents with respect to POL6014 held by Polyphor and certain other parties and exclusively licensed or sublicensed, as applicable, to us, are due to expire in 2025, subject to potential extended market protection. Vamorolone is protected by method of use patents held by ReveraGen for a number of indications, including muscular dystrophy. ReveraGen's method of use patents for the treatment of muscular dystrophy are due to expire in 2029. Further, we may not be able to rely on patent protection for any of our future product candidates.

The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates in certain countries. There is no assurance that all potentially relevant prior art relating to such patents and patent applications has been identified. We may be unaware of prior art that could be used to invalidate an issued patent or prevent pending patent applications from issuing as patents. Even if patents do successfully issue and even if such patents cover our product candidates, third parties may challenge their validity, enforceability or scope, which may result in such patents being narrowed or invalidated. Without extensive patent protection, we will only be able to rely upon the time-limited market exclusivity, if any, resulting from any orphan drug designation, which may be revoked, will only apply for a limited time period and will be subject to other conditions and limitations (see risk factor “*Our business model relies on orphan drug exclusivity for our marketed product, Raxone® in LHON, and most of our current or future clinical product candidates. Orphan drug designation can be difficult to obtain and maintain, and it provides only limited protection from competition.*”). If we are unable to obtain, or if we or our licensors or sublicensors lose, patent protection with respect to any of our products or product candidates, we may be unable to prevent competitors from entering the market with a product that is similar to or the same as our product or product candidate. Further, we may be subject to ownership disputes over patents or other intellectual property with licensors, sublicensors, former employees, collaborators or other third parties.

***We have in-licensed our early stage pipeline and other intellectual property, and have acquired an option to in-license vamorolone, from third parties. We could lose our rights to use the licensed intellectual property in the event of termination of or dispute relating to the relevant license or if such intellectual property is unenforceable for any reason. In addition, enforcement of in-licensed intellectual property and defending against third-party claims in relation thereto are more complex than in the case of our own owned intellectual property.***

We have acquired an exclusive option to obtain from Idorsia an exclusive sub-license to commercialize ReveraGen's vamorolone, subject to an upfront payment of USD 20.0 million to Idorsia, which we intend to make from the proceeds of this Offering, and the issuing of 1,000,000 Idorsia Shares to Idorsia. We will be able to exercise this option against payment of USD 30.0 million at the latest when the data from the Phase IIb study in Duchenne muscular dystrophy (“**DMD**”) patients are available (see “—*License and collaboration agreements*” in “*The Company and its Business*” beginning on page 84 for more information). In addition, we have in-licensed omigapil from Novartis and POL6014 from Polyphor, in each case on an exclusive world-wide basis (see “—*License and collaboration agreements*” in “*The Company and its Business*” beginning on page 84 for more information). The same risks that apply to the intellectual property rights we own generally apply with respect to protection of intellectual property that we license. If we or our licensors fail to prosecute, maintain and enforce such intellectual property or if such intellectual property is unenforceable or if a licensor would enter bankruptcy or similar distressed status, we could lose our rights to use such intellectual property or our exclusivity with respect to those rights. The same may be the case if the agreements by which we have in-licensed or under which we have the option to in-license intellectual property are terminated or if a dispute arises between us and our licensing partners in relation to our

rights or obligations under the license or option agreements, including any such conflict, dispute or disagreement arising from our failure to satisfy payment obligations under any such agreements. In addition, the enforcement of in-licensed intellectual property in case of violations or misappropriation by third parties and defending against third-party claims in relation to in-licensed intellectual property are more complex than in the case of owned intellectual property. Such proceedings may require coordination with the licensor, and licensors typically have rights to intervene or veto rights. As a result of these factors, our ability to develop and commercialize the affected product candidates may be adversely affected and we may not be able to prevent competitors from making, using, and selling competing products.

***Third-party claims of intellectual property infringement or misappropriation may prevent or delay our product development and commercialization efforts.***

There is a substantial amount of litigation involving patent and other intellectual property rights in the pharmaceutical and biotechnology industries, including patent infringement lawsuits, interferences, oppositions and *inter partes* reexamination proceedings before the government patent offices. Numerous patents and pending patent applications owned by third parties exist in the fields in which we are active. Third parties may assert that we infringe their intellectual property, and patent applications covering our product candidates could have been filed by others without our knowledge. We may also face a claim of misappropriation if a third party believes that we inappropriately obtained and used trade secrets of such third party. Parties making claims against us may obtain injunctive or other equitable relief that could effectively prevent us from further developing or commercializing our product candidates or marketing our product or any future products. We have not conducted a freedom-to-operate search or analysis for our own or in-licensed products (including vamorolone). Thus, we may not be aware of third parties' intellectual property that our products, or our sale or commercialization thereof, may infringe or that, if issued, would block us from selling or otherwise commercializing our products. If any third-party patents were held by a court of competent jurisdiction to cover aspects of our materials, formulations, methods of manufacture or methods of treatment, the holder of any such patents would be able to block our ability to develop and commercialize the applicable product candidate until such patent expired or unless we or our partners obtain a license. These licenses may not be available on acceptable terms, if at all. Even if we or our partners were able to obtain a license, the rights may be non-exclusive, which could result in our competitors gaining access to the same intellectual property. Ultimately, we could be prevented from commercializing a product candidate, or be forced to cease some aspect of our business operations, if as a result of actual or threatened patent infringement claims, we or our partners are unable to enter into licenses on acceptable terms. Defending against claims of patent infringement or misappropriation of trade secrets could be costly and time-consuming, regardless of the outcome, and result in significant demands on the time and attention of the management teams. In the event of a successful claim of infringement, we may be required to pay substantial damages, royalties or other financial remedies and incur other significant costs, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible, or require substantial time and monetary expenditure, or incur other significant costs and lose the patent protection to which we thought we were entitled.

During the course of any patent or other intellectual property litigation, there could be public announcements of the results of hearings, rulings on motions, and other interim proceedings in the litigation. If potential and current partners or collaborators or securities analysts or investors regard these announcements as negative, the perceived value of our technology, product candidates and products, development programs or intellectual property could be diminished.

Furthermore, as set out in "X. *The Company and its Business—E. Additional information on our business—5. Market exclusivity and intellectual property*" beginning on page 90, the U.S. government has reserved certain rights to vamorolone. As a consequence, ReveraGen is required to comply with certain formalities, including in particular the filing of certain information with governmental databases. Whether or not ReveraGen complies with this requirement is beyond our control. Should ReveraGen be found to be, or have at any point been, in breach of such filing or other obligations in connection with vamorolone, this could result in the retransfer of intellectual property rights in connection with vamorolone to the U.S. Army Medical Research and Materiel Command (USAMRMC) or any successor or other governmental entity or authority and/or in any of ReveraGen, Idorsia and/or ourselves being involved in a litigation relating to

intellectual property rights in connection with vamorolone, each of which could have a materially adverse effect on our business, results of operations, financial position and cash flows and potentially damage our reputation.

***We enjoy only limited geographical protection with respect to patents and may face difficulties in certain jurisdictions, which may diminish the value of intellectual property rights in those jurisdictions.***

We and our licensors have not filed for patent protection in all national and regional jurisdictions where such protection may be available; instead, we have sought such protection only with respect to jurisdictions that we currently anticipate being key to our business, in particular the U.S. and the EU. In addition, we may decide to abandon national and regional patent applications before grant. Finally, the grant proceeding of each patent in each jurisdiction is an independent proceeding, and applications might in some jurisdictions be refused, while granted in others, which may ultimately limit our ability to rely on jurisdictional exclusivity, if any, for our marketed product or our product candidates in certain jurisdictions. Depending on the jurisdiction, the scope of patent protection may vary for the same product candidate or technology.

The laws of some jurisdictions do not protect intellectual property rights to the same extent as the laws in the U.S. and Europe, and many companies have encountered significant difficulties in protecting and defending such rights in such jurisdictions. If we or our licensors encounter difficulties in protecting, or are otherwise precluded from effectively protecting, the intellectual property rights important for our business in such jurisdictions, the value of these rights may be diminished, and we may face additional competition from others in those jurisdictions.

Many countries have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many countries limit the enforceability of patents against government agencies or government contractors. In these countries, the patent owner may have limited remedies, which could materially diminish the value of such patent. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired and our business and results of operation may be adversely affected.

***If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.***

We rely on trade secrets and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our technology and development processes that involve proprietary know-how, information or technology that is not covered by patents. In addition, we rely on our employees, advisors, third party contractors such as CROs, consultants and collaboration partners to develop and manufacture our product and product candidates, which is why we must, at times, share our intellectual property and trade secrets with them.

Trade secrets can be difficult to protect. We seek to protect our proprietary and in-licensed technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, and outside scientific advisors, contractors and collaborators, but our employees, consultants, outside scientific advisors, contractors and collaborators may intentionally or inadvertently disclose our trade secret information to competitors. In addition, our competitors may gain access to our trade secrets through legal or illegal means or independently develop substantially equivalent information and techniques. We may not be able to protect trade secrets effectively and we may not have adequate remedies against misappropriation of trade secrets. Enforcing a claim that a third party illegally obtained and is using any of our trade secrets is expensive and time consuming and the outcome is unpredictable. In addition, courts outside the U.S. sometimes are less willing than U.S. courts to protect trade secrets. Misappropriation, unauthorized disclosure or a competitor's discovery of our trade secrets could materially impair our competitive position or our business.

Many of our employees were previously employed at universities or other pharmaceutical companies, including our competitors or potential competitors. We may be subject to claims that we or our employees

have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of our employees' former employers or other third parties. We may also be subject to ownership disputes in respect of intellectual property created by these employees during the course of their employment with us. Any of such claims could result in our competitive position being impaired and our business and results of operation may be adversely affected.

***We may become involved in lawsuits to protect or enforce our patents and other exclusivity rights, which could be expensive, time-consuming, and unsuccessful.***

Competitors may infringe our intellectual property, the intellectual property of our licensors, or the market exclusivity resulting from orphan drug designations that we have achieved. To counter or defend against such claims can be expensive and time-consuming. In an infringement proceeding, a court may decide that a patent owned or in-licensed by us is invalid or unenforceable and/or may refuse to stop the other party from using the technology at issue. An adverse result in any litigation over exclusivity rights could put one or more of our or our licensors' patents at risk of being invalidated or interpreted narrowly or an orphan drug designation of being revoked. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation in many jurisdictions, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

We may also be subject to claims that former employees, collaborators or other third parties have an ownership interest in the patents or other intellectual property we own or license-in. We may be subject to ownership disputes in the future arising from, for example, conflicting obligations of consultants or others who are involved in developing our product candidates. Litigation may be necessary to defend against these and other claims challenging inventorship or ownership. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of, or right to use, valuable intellectual property. Such an outcome could have a material adverse effect on our business. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and employees.

## **6. Risks related to manufacturing, employment matters, operations, managing growth, corporate structure and financial reporting**

***We have no manufacturing capabilities or capacity of our own and rely on third parties for production of Raxone<sup>®</sup> and our other compounds, omigapil and POL6014.***

We have no manufacturing capabilities or capacity of our own and have outsourced the entire manufacture, formulation, packaging, storage and distribution of Raxone<sup>®</sup> and our other compounds, omigapil and POL6014, to third parties. We currently have no plans to build up or acquire manufacturing capacity and the related know-how of our own in relation to omigapil and POL6014.

For the production of Raxone<sup>®</sup>, we rely on a drug substance supplier, with whom we have agreed on a seven-year exclusivity period (subject to exceptions) starting with the first launch of Raxone<sup>®</sup> in Europe and the U.S., respectively. In Europe, such exclusivity period will lapse in October 2022. We currently have one finished drug product supplier of Raxone<sup>®</sup>. If any of our manufacturing agreements is terminated or not renewed by the third-party provider, we may not be able to timely negotiate a new agreement with that or another third-party provider on acceptable terms or at all. Furthermore, switching a supplier of the drug substance or the finished drug product is an expensive and time-consuming process.

We rely on our licensor, Novartis, to provide omigapil. We primarily rely on Polyphor to supply the active pharmaceutical ingredient of the POL6014 compound. To the extent we may not be able to use Polyphor's inventory of the active pharmaceutical ingredient of the POL6014 compound, we rely on a third-party manufacturer of the POL6014 compound. We also rely on PARI Pharma GmbH, Gräfelfing, Germany, as the manufacturer of the nebulizer called eFlow<sup>®</sup> with which POL6014 is administered.

The facilities used by our suppliers to manufacture Raxone<sup>®</sup> and the finished products containing omigapil or POL6014 are subject to approval and inspections by regulatory authorities. We do not have full control

over our suppliers' quality control or compliance with laws, regulations or cGMP standards, and any non-compliance could result in sanctions being imposed also on us, including fines, injunctions, civil penalties, delays, suspension, withdrawal or non-grant of market of approvals, seizures or recalls of products, operating restrictions and criminal prosecutions.

***The compounds we use are complex and difficult to manufacture. Only a handful of manufacturers are able to manufacture these compounds, and our manufacturers may experience production problems.***

The manufacturing of our compounds necessitates compliance with regulatory requirements, such as cGMP, and is complex, time-consuming and expensive. In particular, only a handful of manufacturers are able to manufacture idebenone in compliance with all regulatory requirements. Manufacturing idebenone involves heavy metal catalysts, the incomplete removal of which in the manufacturing process would result in toxic amounts of these impurities remaining in the drug substance, and non-cGMP synthesis of idebenone may result in other toxic or cancerogenic by-products. Problems with the manufacturing process, even minor deviations from the normal process, could result in contamination, product defects or manufacturing failures that could result in harm to patients, lot failures, product recalls, product liability claims, or insufficient inventory. Regulatory authorities may require us to submit samples of any lot or may require that we do not distribute a lot until the agency authorizes its release. Our contract manufacturers may be unable to achieve adequate quantities and quality of clinical-grade materials, and their supply chain could be interrupted from time to time. Any such problems could materially harm our business, financial condition, results of operations, and prospects.

***If we lose the services of any member of our top management or other key members of our management, scientific or commercial staff, or if we fail to attract and retain key scientific or other personnel, we may be unable to successfully develop and commercialize our product candidates or market our current marketed product or any future products for which we obtain marketing authorization.***

We are highly dependent on the performance and expertise of members of our top management, especially our CEO, whose responsibilities include those of a Chief Scientific Officer, and other key members of our management, scientific and commercial staff. We are a small company with many key functions being carried out by one person only. The loss of the services of any of our key personnel for any reason or our inability to attract new highly qualified and experienced employees could harm our business. Furthermore, we do not currently maintain "key person" insurance for any of our executives or other employees.

A limited number of people have experience and know-how in neuro-ophthalmologic, neuromuscular and pulmonary diseases and the product and product candidates developed by us. To foster retention, we have established employee participation plans, but there is intense competition for skilled personnel. If our product candidates are granted marketing authorizations or if we expand our development activities, we would need to hire additional personnel, which may be difficult to recruit and retain on acceptable terms given such competition.

***We will need to grow the size and capabilities of our organization, and we may experience difficulties in managing this growth.***

As of September 30, 2018, we had 119 employees (113.6 full-time equivalent) and we expect our headcount to increase in the near future in connection with the further rollout of Raxone<sup>®</sup> in LHON in the EU, as a result of the ramp up of our activities in the U.S., and as a consequence of our planned clinical development of POL6014. Our future financial performance will depend, in part, on our ability to effectively manage any future growth. We will need to expand and effectively manage our organization, personnel, operations and facilities in order to successfully develop and commercialize our marketed product and our product candidates. We will only be able to organize operations efficiently and avoid a misallocation of resources if we continue to improve our operational, financial and management controls, reporting systems and procedures. Our management may have to divert a disproportionate amount of its attention away from day-to-day activities in order to manage these growth activities. If we are unable to effectively expand our organization, we may not achieve our development and commercialization goals and our operational efficiency may be materially adversely affected.

***Our and our partners' computer systems may fail or suffer security breaches, which could result in a material disruption of our product development programs and our business operations.***

Despite the implementation of security measures, our computer systems and those of our current and any future suppliers, CROs and other contractors, consultants and collaborators are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our product development programs and our business operations, whether due to a loss of our trade secrets or other disruptions. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs due to efforts to recover or reproduce the data. To the extent that any disruption or security breach were to result in data loss or inappropriate disclosure of confidential or proprietary information or personal data of patients or other persons, we may be exposed to liability and suffer reputational damage.

## **7. Risks related to general economic and financial market conditions**

***Changes in the macro-economic environment and political developments in Europe, the United States and elsewhere may have a material adverse effect on the Group and may reduce the value of our Shares.***

Over the past years, there has been a series of political and economic events such as the past global economic financial crisis, sovereign debt and financial crises in several EU countries, the decision of the United Kingdom to leave the EU (commonly known as “Brexit”), the conflict in Syria, the 2016 U.S. presidential election and increased tensions in the Middle East. These events have impacted the global economy at large, the economies and financial situation of governments in many of our current and potential future markets, as well as exchange rates (in particular the euro/Swiss franc rate) and have been associated with, among other things, instability of financial institutions, high market volatility, liquidity problems, limited availability of financing and legal uncertainty. Recession or rising inflation and other effects may also be a consequence of these events. This uncertain macro-economic environment may have a material adverse effect on our business, results of operations, financial condition, prospects, or the market price of our securities, including our Shares.

***We are exposed to currency fluctuation risks and other financial risks.***

While we incur costs mainly in Swiss francs, a significant proportion of our costs are required to be paid in euros. To the limited extent we generate sales, we receive payments primarily in euros. If and as our business grows, we expect that a significant part of our sales and a significant part of our expenses will be denominated in euros. Our reporting currency is the Swiss franc and, as a result, financial line items are converted into Swiss francs at applicable foreign exchange rates. Further, we are subject to interest rate risks. Unfavorable developments in the value of the Swiss franc as compared to the euro, in interest rates and in the capital markets could have a material adverse effect on our financial condition and results. For a discussion of our foreign exchange rate, interest rate, credit and liquidity risks please also refer to pages 53 through 56 of our 2017 Consolidated Financial Statements included elsewhere in this Offering Memorandum.

## **B. Risks related to the Offering and the Shares**

***We have broad discretion in the use of the net proceeds from the Offering and may not use them effectively.***

The Company intends to use the equivalent of USD 20.0 million of the net proceeds from the Offering to pay the cash component of the consideration to Idorsia for the acquisition of the option for the exclusive sub-license relating to ReveraGen's vamorolone. The Company intends to use any net proceeds in excess of that amount for general corporate purposes, including but not limited to for potential capital expenditures, general research and development and/or the acquisition or in-licensing of additional compounds, product candidates, technology or businesses. The Company will have broad discretion in the allocation and use of

any net proceeds from the Offering that it intends to apply for general corporate purposes and our ultimate use of such net proceeds may differ substantially from their currently intended use. A failure to use the net proceeds from the Offering effectively could adversely affect the Group's business, financial condition, results of operations or prospects.

***Shareholders may suffer dilution as a result of further issuance of equity, conversions of our Bonds or further issuances of other securities convertible into equity.***

On December 11, 2018, the shareholders of the Company resolved on an ordinary capital increase of up to CHF 5,000,000 by issuing a maximum of 5,000,000 new shares in the Company with a par value of CHF 1 each. The Company intends to use the net proceeds of this ordinary capital increase to fund the USD 20.0 million payable to Idorsia as cash consideration for the acquisition of the option for the exclusive sub-license relating to ReveraGen's vamorolone and any net proceeds from the Offering in excess of that amount for general corporate purposes. In addition, we may need or elect to raise additional equity or equity-linked financing in the future in order to continue our operations as planned.

In addition, holders of our Bonds have the right to convert the Bonds before their maturity in 2022 into an aggregate of up to 925,926 Shares at a conversion price of CHF 64.80 per Share. Also, the Company has issued, and may issue in the future, other rights to acquire Shares. As of September 30, 2018, such rights to acquire 268,799 Shares (excluding conversion rights under the Bonds) were outstanding (see "*Options, warrants and conversion rights*" in "*Description of the Company's Capital Structure and Shares*" beginning on page 136).

When the capital increase is implemented, our existing shareholders will incur substantial dilution. Additional dilution may occur to the then-existing shareholders if and to the extent that the Bonds will be converted and such rights to acquire Shares will ultimately be exercised and settled in Shares. Moreover, to the extent that the Company issues additional shares or equity-linked instruments (*e.g.*, for financing purposes or for employee participations), investors' ownership interest will be further diluted, and the terms of such issued shares may include liquidation or other preferences that adversely affect investors' rights as a shareholder.

***The Share price has been and is expected to be volatile, and investors may not be able to resell their Shares at or above the Offer Price.***

The market price of the Shares has historically been subject to substantial fluctuations. We expect the market price of the Shares to continue to be highly volatile. Such volatility may depend upon many factors within and beyond our control, including the risk factors listed in this Offering Memorandum, our or our competitors' financial and business performance, general market conditions and the volatility in financial and other markets (*i.e.*, the degree to which prices fluctuate over a particular period in a particular market, regardless of market levels) in general. In some cases, the markets have produced downward pressure on share prices for certain issuers seemingly without regard to those issuers' underlying financial strength. Also, the Offer Price will be determined following an accelerated bookbuilding process and may not be indicative of the price at which the Shares will trade after completion of the Offering. As a result, the Shares may trade at prices significantly below the Offer Price.

***The trading market for the Shares is not liquid and shareholders may not trade or sell their Shares easily or at all.***

The volume of the trading market for the Shares on the SIX Swiss Exchange has been low and is expected to be low in the future. Therefore, the trading market may not provide enough liquidity to allow shareholders to trade or sell their Shares easily or at all. The Company is not obliged to provide a bid or offer price for the Shares. Further, the Company's market making arrangement with Kepler Cheuvreux SA may be terminated at any time, and even while this arrangement is in place, there is no assurance that shareholders will be able to trade or sell their Shares easily or at all.

***Future sales of a substantial number of Shares or derivative instruments by us or our investors could adversely affect the market price of the Shares.***

Sales, or the possibility or perceived possibility of sales, of a substantial number of Shares in the market could have a material adverse effect on the market price of the Shares. Idorsia has agreed with the Company that, without the prior written consent of the Company and subject to exceptions, it will not, until the Company obtains FDA approval for the sale of vamorolone in DMD, sell any Idorsia Shares in the public market or effect certain other transactions in the Idorsia Shares or related to the Idorsia Shares (see “—Lock-up” in “*The Issuance of the New Shares*” beginning on page 117 for more details). Further, the Company has agreed with the Managers to a 90-day lock-up commencing on the date of the Accelerated Book-Build Agreement with respect to certain transactions in Shares and share-based instruments. See “—Lock-up arrangements” in “*Offering and Sale*” beginning on page 147. Other than that, none of the shareholders of the Company is bound by a lock-up agreement. As is the case for any other shareholders not subject to a lock-up agreement, investors who have purchased Shares in the Offering may sell some or all of their Shares in the open market immediately after the completion of the Offering. Also, holders of Bonds have the right to convert the Bonds into Shares at any time before the maturity of the Bonds in 2022 and will be able to sell some or all of the Shares issued by the Company upon such conversion in the open market at any time. In addition, the Company may issue additional Shares out of its existing authorized share capital or may propose to its shareholders to approve additional capital increases, in each case excluding shareholders’ preemptive rights. As a result of the respective issuances or sales of Shares, or if such issuances or sales are anticipated by investors, the market price of the Shares could fall substantially.

***The Company does not expect to pay dividends in the foreseeable future.***

Since its inception, the Company has never paid any dividends and it does not anticipate paying dividends in the foreseeable future. Investors cannot rely on dividend income from the Shares, and any returns on an investment in the Shares will likely depend entirely upon any future appreciation in the price of the Shares and the ability of investors to sell Shares in the market.

***Shareholders outside Switzerland may not be able to exercise preemptive rights in future issuances of equity or other securities that are convertible into equity.***

Under Swiss law, shareholders may have certain preemptive rights to subscribe on a pro rata basis for issuances of newly issued equity or other securities that are convertible into equity. Due to laws and regulations in their respective jurisdictions, non-Swiss shareholders may not be able to exercise such rights unless we take action to register or otherwise qualify the rights offering under the laws of that jurisdiction. There can be no assurance that we would take any such action, and we will have the full discretion to decide not to take such action in one or more jurisdictions, including the EU and the U.S. If shareholders in such jurisdictions are unable to exercise their subscription rights, their ownership interest in the Company would be diluted.

***Shareholders may face additional investment risk from currency exchange rate fluctuations in connection with their holding of Shares.***

The Shares are and will be quoted in Swiss francs only, and future dividends, if any, will be denominated in Swiss francs. If the Swiss franc depreciates against a foreign currency that is the main currency of a shareholder, the value of the Shares or of any dividend, expressed in such foreign currency, will decrease accordingly. Prospective investors should be aware that exchange rates between currencies are highly volatile. Foreign exchange fluctuations between a shareholder’s main currency and the Swiss franc may adversely affect shareholders who intend to convert the proceeds from the sale of the Shares or future dividends, if any, into their main currency and may potentially cause a partial or total loss of a shareholder’s initial investment.

***If securities or industry analysts do not publish research at all or publish inaccurate or unfavorable research about the Group's business, the market price and/or the trading volume of the Shares could decline.***

The trading market for the Shares depends in part on the research and reports that securities or industry analysts publish about the Group or its business. If no or few securities or industry analysts cover the Company, the market price for the Shares could be adversely affected. If one or more of the analysts who cover the Group downgrades a recommendation with regard to the Shares, publishes inaccurate or unfavorable research about the Group's business, ceases to cover the Group or fails to publish reports on it regularly, the market price and/or the trading volume of the Shares would likely decline.

***Our largest shareholders are able to exert influence over the Company, and their interests may not necessarily be the same as those of other shareholders.***

As of the date of this Offering Memorandum, the Company's largest shareholder is Idorsia who owns an aggregate of 13.3% of the voting rights in the Company. Idorsia and our other significant investors together own an aggregate of 32.26% of the voting rights in the Company. Any of our investors may start acting in concert or may acquire significant ownership interests in the Company in the future. Such shareholders or groups of shareholders may be able to exert influence over, and potentially block, certain matters that must be decided by the Company's general meeting of shareholders, in particular those matters that require the consent of two-thirds of voting rights represented (see "*—General meeting of shareholders*" in "*Description of the Company's Capital Structure and Shares*" beginning on page 139 for more information on majority requirements). The influence of significant shareholders or groups of shareholders is accentuated by the low historic rates of participation at the Company's past three annual general meetings of shareholders, which were between 33.3% (2018) and 51.2% (2016). The interests of influential shareholders may not be the same as the interests of the Company's other shareholders, and respective corporate decisions may materially adversely affect the interests of the other investors in the Company.

***Our articles of association provide for an opting out of the mandatory tender offer rules. As a result, our shareholders would not have the possibility to sell their Shares in the event that a shareholder or group of shareholders acquires more than 33 1/3% of the voting rights in the Company. Also, the minimum price rules would not be applicable in any voluntary public tender offer for Shares in the Company.***

Our articles of association exempt shareholders from the mandatory tender offer rules under the Federal Act on Financial Market Infrastructures and Market Conduct in Securities and Derivatives Trading of June 19, 2015 (the "**FMIA**") and its predecessor. As a result, any shareholder or group of shareholders exceeding the threshold of 33 1/3% of the voting rights (whether exercisable or not) of the Company would not be required to make a mandatory public tender offer for all Shares. Accordingly, our shareholders would not have the possibility to sell their Shares in the event that a shareholder or group of shareholders obtains control of the Company. Also, voluntary public tender offers may be made at less than the minimum price under the mandatory tender offer rules (*i.e.*, the higher of the pre-offer 60-day volume weighted average price and the highest price paid by the offeror for equity securities within the last 12 months) even if the offeror would, as a result, hold more than 33 1/3% of the voting rights in the Company.

***The Offering may not be completed for various reasons.***

The Offering may not be completed if certain conditions and representations by the Company contained in the Accelerated Book-Build Agreement are not satisfied or are breached, respectively (see "*—Placement of Offered Shares*" in "*Offering and Sale*" beginning on page 147 for more information on the Accelerated Book-Build Agreement). If one or more of such conditions were not satisfied, or if there were a breach of any such representations, the Offering may be terminated by the Managers at any time prior to its completion. In such an event, the Offering becomes void and any Share trades effected before the final settlement will not be honored.

***The shareholders' resolution regarding the capital increase may be challenged.***

The Offering is based upon the resolution regarding an ordinary capital increase approved by the extraordinary shareholders' meeting held on December 11, 2018. As with all shareholders' resolutions of Swiss corporations, such a resolution is subject to a possible challenge pursuant to articles 706 and 706a CO. In connection with such a challenge, the registration of the capital increase in the commercial register may be blocked and, therefore, prevent or delay the completion of the Offering, including a delay in the settlement of the Offered Shares. Consequently, prospective investors may suffer losses, in particular, if they entered into short selling transactions and are unable to meet their obligations to deliver Offered Shares.

***It is not certain that the increase of the share capital in connection with the Offering will occur when anticipated.***

Although the resolution of the shareholders to increase the share capital is scheduled to be registered with the commercial register in a timely manner, such registration may, for reasons beyond our control, not take place in time to enable the Offered Shares to be traded commencing on or about the First Day of Trading.

***If the Offering is not completed, we will not be able raise the necessary funds for the payment of the cash consideration regarding the assignment of the option on vamorolone; however Idorsia will keep the 1,000,000 Idorsia Shares.***

The Company intends to use the proceeds of the ordinary capital increase to fund the USD 20.0 million payable to Idorsia as cash consideration for the acquisition of the option for the exclusive sub-license relating to vamorolone. If the Offering is not completed, we will not be able to fund such a payment. However, the 1,000,000 Shares issued to Idorsia will not be returned or rescinded in case that we are unable to finance the USD 20.0 million cash portion of the acquisition consideration and the acquisition therefore will not close. This would lead to a substantial dilution of the existing shareholders without specific counter value.

***U.S. shareholders may not be able to obtain judgments or enforce civil liabilities against the Company or its directors or executive officers.***

The Company is organized under the laws of Switzerland and has its seat in the Canton of Basel-Landschaft, Switzerland. Most of its assets are located outside the U.S. In addition, none of its directors and executive officers are residents of the U.S. and all of or a substantial portion of their assets are located outside the U.S. As a result, it may not be possible for investors to effect service of process within the U.S. upon the Company or such persons or to enforce against them judgments of U.S. courts, including judgments in actions predicated upon the civil liability provisions of the federal securities laws of the U.S. We have been advised by our Swiss counsel that there is doubt as to the enforceability in Switzerland of original actions, or in actions for enforcement of judgments of U.S. courts, of civil liabilities to the extent predicated upon the federal and state securities laws of the U.S. Original actions against persons in Switzerland based solely upon the U.S. federal or state securities laws are governed, among other things, by the principles set forth in the Swiss Federal Act on International Private Law of December 18, 1987, as amended ("**PILA**"). This statute provides that the application of provisions of non-Swiss law by the courts in Switzerland shall be precluded if the result is incompatible with Swiss public policy. Also, mandatory provisions of Swiss law may be applicable regardless of any other law that would otherwise apply.

Switzerland and the U.S. do not have a treaty providing for reciprocal recognition and enforcement of judgments in civil and commercial matters. The recognition and enforcement of a judgment of the courts of the United States in Switzerland is governed by the principles set forth in the PILA. This statute provides in principle that a judgment rendered by a non-Swiss court may be enforced in Switzerland only if (i) the non-Swiss court had jurisdiction pursuant to the PILA; (ii) the judgment of such non-Swiss court has become final and non-appealable; (iii) the judgment does not contravene Swiss public policy; (iv) the court procedures and the service of documents leading to the judgment were in accordance with the due process of law; and (v) no proceeding involving the same position and the same subject matter was first brought in Switzerland, or adjudicated in Switzerland, or was earlier adjudicated in a third state and this decision is recognizable in Switzerland.

***If the Company is classified as a passive foreign investment company for U.S. federal income tax purposes, U.S. investors that hold the Company's shares could be subject to potentially significant adverse tax consequences.***

If the Company is classified as a passive foreign investment company (“PFIC”) in any taxable year in which a U.S. Holder, as defined in “—U.S. federal income taxation of Offered Shares” in “Taxation” beginning on page 159, holds Shares, such U.S. Holder may be subject to significant adverse tax consequences. The Company has not determined whether it was a PFIC for its taxable year ended December 31, 2017, and, because the factual elements underlying this analysis are subject to change (in particular based on fluctuations in market conditions), and because the interpretation of the law relating to PFIC status is not clear in all respects, the Company cannot provide assurances as to whether it will be classified as a PFIC in the current taxable year or in the future.

The Company will be classified as a PFIC in respect of any taxable year in which, after taking into account its income and gross assets (and the income and assets of certain affiliates pursuant to applicable “look-through rules”) either (i) 75% or more of its gross income consists of certain types of “passive income” or (ii) 50% or more of the average quarterly value of its assets is attributable to “passive assets” (assets that produce or are held for the production of passive income). Passive income generally includes interest, dividends, rents, certain non-active royalties and capital gains. PFIC status is a factual determination that needs to be made annually after the close of each taxable year, on the basis of the composition of the Company’s income and assets, the relative value of its active and passive assets from time to time, and its market capitalization. For this purpose, certain of the Company’s assets are treated as passive even though it holds them in the ordinary course of its business operations.

The composition of the Company’s income and assets will be affected by how, and how quickly, the Company uses the proceeds from this Offering. Under circumstances where the cash is not deployed for active purposes, the Company’s risk of becoming a PFIC may increase. Because (i) the Company currently owns, and will own after the completion of the Offering, a substantial amount of passive assets, including cash, and (ii) the values of the Company’s assets, including intangible assets, are uncertain and may vary substantially over time, there is a material risk that the Company will be, and there can be no assurance that the Company will not be, a PFIC in 2018 or any future year.

If the Company were to be classified as a PFIC, a U.S. Holder that does not make a “mark-to-market” election may incur significantly increased U.S. income tax on gain recognized on the sale or other disposition of the Company’s Shares and on the receipt of distributions on the Shares to the extent such distribution is treated as an “excess distribution” under the U.S. federal income tax rules. Additionally, if the Company were to be or become classified as a PFIC, a U.S. Holder of its Shares may be subject to additional U.S. tax form filing requirements, and the statute of limitations for collections may be suspended if the Holder does not file the appropriate form. See “—U.S. federal income taxation of Offered Shares—Passive foreign investment company rules” in “Taxation” beginning on page 159.

## V. USE OF PROCEEDS

The Company targets gross proceeds of approximately CHF 50 million from the sale of up to 5,000,000 new Shares offered in the Offering (based on the maximum number of Offered Shares and the closing price of the Shares on the SIX Swiss Exchange on December 11, 2018), which would result in approximately CHF 46.0 million in net proceeds after deducting estimated underwriting commissions, estimated offering expenses payable by the Company and the Swiss federal issue stamp duty (*Emissionsabgabe*).

The Company intends to use the equivalent of USD 20.0 million of the net proceeds to pay the cash component of the consideration to Idorsia for the acquisition of the option for the exclusive sub-license relating to ReveraGen's vamorolone. The Company intends to use any net proceeds in excess of that amount for general corporate purposes, including but not limited to for potential capital expenditures, general research and development and/or the acquisition or in-licensing of additional compounds, product candidates, technology or businesses. The Company intends to complete the Offering as long as the net proceeds exceed the CHF equivalent of USD 20 million.

The Company will have broad discretion in the use of any net proceeds of the Offering that it intends to apply for general corporate purposes. The expected use of such net proceeds described above represents the Company's intentions based upon its current plans and business conditions, which could change in the future as its plans and business conditions evolve. Except for the portion of the net proceeds to be used to pay to Idorsia the cash component of the consideration for the acquisition of the option for the exclusive sub-license relating to ReveraGen's vamorolone, we cannot currently allocate specific percentages of the net proceeds that we may use for the purposes specified above.

Pending its use of any net proceeds from the Offering for general corporate purposes, the Company intends to invest the net proceeds in a variety of capital preservation instruments, including short-term, investment-grade, interest-bearing instruments and risk free money market instruments.

## VI. DIVIDENDS AND OTHER DISTRIBUTIONS

Since its inception, the Company has never paid any dividends. The Company currently intends to retain all available funds and future earnings, if any, primarily to fund the development and commercialization of Raxone® and its product candidates or for other corporate purposes (see “Use of Proceeds” beginning on page 37). Therefore, the Company does not anticipate paying dividends or other distributions in the foreseeable future. See also risk factor “*The Company does not expect to pay dividends in the foreseeable future.*” beginning on page 33. As a result, investors in Shares will benefit in the foreseeable future only if the Shares appreciate in value.

In order for the Company to declare and pay distributions, the distribution must be approved by shareholders holding an absolute majority of the Shares represented at the general meeting of shareholders. The Company’s board of directors (the “**Board**”) may propose distributions in the form of a dividend or in the form of a distribution of cash or property that is based upon a reduction of the Company’s share capital recorded in the commercial register.

Ordinary dividends may be paid only if the Company has sufficient distributable profits from previous years or freely distributable reserves to allow the distribution of a dividend, in each case, as presented on the Company’s annual statutory standalone balance sheet prepared in accordance with Swiss corporate law. A confirmation by the Company’s auditors is required that a proposal made by the Board to shareholders regarding the appropriation of the Company’s available earnings conforms to the requirements of the CO and the Company’s articles of association. Furthermore, in order for the Company to pay dividends to its shareholders out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*), it is required that a general meeting of shareholders approves, by the absolute majority of votes represented at the meeting, the reclassification of such reserves from capital contributions (*Reserven aus Kapitaleinlagen*) to freely distributable reserves (to the extent permissible by the CO). As of September 30, 2018, the Company had reserves from capital contributions (*Reserven aus Kapitaleinlagen*) in the amount of CHF 449.9 thousand, all of which were freely distributable. A distribution of cash or property that is based upon a reduction of the Company’s share capital requires a special audit report confirming that the claims of the Company’s creditors remain fully covered by the Company’s assets despite the reduction in the share capital. After the general meeting of shareholders has approved the capital reduction, the Board has to give public notice of the capital reduction in the Swiss Official Gazette of Commerce (*Schweizerisches Handelsamtsblatt*) three times and notify the Company’s creditors that they may request, within two months after the third publication, satisfaction of or security for their claims.

All Shares are equally entitled to dividends and other distributions paid by the Company, if any. Dividends and other cash distributions of the Company, if any, will be declared and are expected to be paid in Swiss francs. Holders of Shares will be entitled to any declared and paid dividends after delivery of such Shares to them. For more information on dividends and other distributions see “—*Dividends and other distributions*” in “*Description of the Company’s Capital Structure and Shares*” beginning on page 141. Under the terms of the Bonds, dividends and other distributions would trigger an adjustment of the conversion price (see “—*CHF 60 million Senior Unsecured Convertible Bonds 2017-2022*” in “*The Company and its Business*” beginning on page 88 for more information on the Bonds).

Dividends paid on Shares are subject to Swiss federal withholding tax, except if paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*). Distributions of cash or property that are based upon a capital reduction are not subject to Swiss federal withholding tax. See “—*Swiss taxation*” in “*Taxation*” beginning on page 156 for a summary of certain Swiss tax consequences regarding dividends and other distributions to holders of the Shares.

## VII. CAPITALIZATION AND INDEBTEDNESS

The following table sets forth our cash and cash equivalents and our capitalization on a consolidated basis as of September 30, 2018:

- on an actual basis according to our Unaudited Interim Condensed Consolidated Financial Statements;
- on an adjusted basis reflecting (i) the issuance of the Idorsia Shares on November 21, 2018, (ii) the issuance and sale of the maximum number of 5,000,000 Offered Shares at an assumed offer price of CHF 9.90 per Offered Share (being the closing price of the Shares on the SIX Swiss Exchange on December 11, 2018), (iii) the payment of the USD 20.0 million cash component of the consideration to Idorsia for the acquisition of the option for the exclusive sub-license to ReveraGen’s vamorolone and (iv) the payment of estimated fees and expenses of the Offering.

You should read the following table in conjunction with “*Management’s Discussion and Analysis of Financial Condition and Results of Operations*” beginning on page 43, “*Selected Financial Data*” beginning on page 40 and the Unaudited Interim Condensed Consolidated Financial Statements included elsewhere in this Offering Memorandum.

in CHF thousands	As of September 30, 2018	
	Actual	As adjusted <sup>(1)</sup>
<b>Cash and cash equivalents</b> .....	<b>19,654</b>	<b>46,108</b>
Current liabilities (all unsecured).....	11,828	11,828
Non-current liabilities (all unsecured) .....	62,141	62,141
<b>Total liabilities</b> .....	<b>73,969</b>	<b>73,969</b>
Share capital .....	6,528	12,528
Capital reserves and share premium .....	403,159	453,059
Retained earnings .....	(400,004)	(400,004)
Employee benefit reserve .....	(2,969)	(2,969)
Treasury shares .....	(1,145)	(1,145)
Other components of equity.....	(766)	(766)
<b>Total equity</b> .....	<b>4,803</b>	<b>60,703</b>

- (1) Adjustments comprise (i) the issuance of the Idorsia Shares on November 21, 2018, resulting in an increase in share capital of CHF 1.0 million and an increase in capital reserves and share premium estimated at CHF 8.9 million based on the closing price of the Shares on the SIX Swiss Exchange of CHF 9.90 per Share on December 11, 2018, (ii) the issuance and sale of the maximum number of 5,000,000 Offered Shares at an assumed offer price of CHF 9.90 per Offered Share (being the closing price of the Shares on the SIX Swiss Exchange on December 11, 2018), which would result in CHF 46.0 million in net proceeds (after deducting estimated offering commissions and expenses payable by the Company and the Swiss federal issue stamp duty (*Emissionsabgabe*)) and increases in share capital of CHF 5.0 million and capital reserves and share premium of CHF 41.0 million and (iii) the payment of the USD 20.0 million cash component of the consideration to Idorsia for the acquisition of the option for the exclusive sub-license to ReveraGen’s vamorolone, translated into Swiss francs at an exchange rate of 0.9773 Swiss franc per USD, which is the exchange rate as of September 30, 2018 used in the preparation of our Unaudited Interim Condensed Consolidated Financial Statements. Our actual capitalization after completion of the Offering will depend on the final number of Offered Shares sold in the Offering and the final Offer Price, and the contribution of the issuance of the Idorsia Shares to our capital reserves and share premium can only be finally determined when the valuation of the related asset is completed following the payment of the USD 20.0 million cash component to Idorsia. Accordingly, our cash and cash equivalents and total equity could be substantially lower than the amounts set forth above if the actual number of Offered Shares sold and/or the final Offer Price are lower than the assumed amounts set forth above.

As of the date of this Offering Memorandum, there have been no changes to the information set forth in the table above, other than (i) as a result of ongoing normal operating activities, such as changes in the cash and cash equivalents and results of operations of the Company, (ii) as otherwise discussed in this Offering Memorandum and (iii) any changes that would not have a material adverse effect on the Company.

## VIII. SELECTED FINANCIAL DATA

Unless otherwise stated, the selected historical financial information presented below as of and for the years ended December 31, 2015, 2016 and 2017 has been extracted or derived from the Audited Consolidated Financial Statements and the selected financial information presented below as of and for the nine months ended September 30, 2017 and 2018 has been extracted or derived from the Unaudited Interim Condensed Consolidated Financial Statements, all of which are included elsewhere in this Offering Memorandum.

The selected historical financial information presented below should be read in conjunction with, and is qualified in its entirety by reference to, the Audited Consolidated Financial Statements and the Unaudited Interim Condensed Consolidated Financial Statements, respectively.

### A. Selected financial information from the consolidated statement of comprehensive income

in CHF thousands	For the financial year ended December 31,			For the nine months ended September 30,	
	2015	2016	2017	2017	2018
	<i>(audited)</i>			<i>(unaudited)</i>	
<b>Net sales</b> .....	<b>4,321</b>	<b>19,033</b>	<b>22,943</b>	<b>16,347</b>	<b>23,634</b>
Cost of goods sold.....	(1,371)	(3,883)	(4,104)	(3,028)	(3,606)
<i>Of which amortization intangible asset</i> .....	<i>(1,013)</i>	<i>(3,039)</i>	<i>(3,039)</i>	<i>(2,279)</i>	<i>(2,279)</i>
Other operating income.....	188	361	270	243	1
Development.....	16,651	(17,675)	(26,561)	(18,168)	(27,098)
<i>Of which development expenses</i> .....	<i>(10,453)</i>	<i>(17,675)</i>	<i>(26,561)</i>	<i>(18,168)</i>	<i>(27,098)</i>
<i>Of which reversal impairment on intangible assets and inventory</i> .....	<i>27,104<sup>(1)</sup></i>	<i>0</i>	<i>0</i>	<i>0</i>	<i>0</i>
Marketing and sales.....	(8,356)	(21,051)	(28,522)	(19,909)	(18,637)
General and administrative.....	(8,244)	(9,805)	(14,416)	(9,573)	(11,292)
Other operating expenses.....	(16)	(107)	(64)	(68)	(169)
<b>Operating expenses</b> .....	<b>35</b>	<b>(48,638)</b>	<b>(69,563)</b>	<b>(47,718)</b>	<b>(57,196)</b>
<b>Operating result</b> .....	<b>3,173</b>	<b>(33,127)</b>	<b>(50,454)</b>	<b>(34,156)</b>	<b>(37,167)</b>
Financial income.....	416	928	4,134	3,533	2,856
Financial expenses.....	(655)	(995)	(4,955)	(3,605)	(5,232)
<b>Result before taxes</b> .....	<b>2,934</b>	<b>(33,194)</b>	<b>(51,275)</b>	<b>(34,228)</b>	<b>(39,543)</b>
Income taxes.....	3,015	(2,221)	(257)	895	(380)
<b>Net result</b> .....	<b>5,949</b>	<b>(35,415)</b>	<b>(51,532)</b>	<b>(33,333)</b>	<b>(39,923)</b>
Items never to be reclassified to net income in subsequent periods					
<i>Actuarial gains (losses) on defined benefit plans</i> .....	<i>(1,671)</i>	<i>(1,776)</i>	<i>(171)</i>	<i>137</i>	<i>1,936</i>
Items to be reclassified to net income in subsequent periods					
<i>Currency translation differences</i> .....	<i>(16)</i>	<i>(18)</i>	<i>82</i>	<i>60</i>	<i>(52)</i>
<b>Other comprehensive result</b> .....	<b>(1,687)</b>	<b>(1,794)</b>	<b>(89)</b>	<b>197</b>	<b>1,884</b>
<b>Total comprehensive result</b> .....	<b>4,262</b>	<b>(37,209)</b>	<b>(51,621)</b>	<b>(33,136)</b>	<b>(38,039)</b>

(1) As a result of receiving marketing authorization in the EU for Raxone® for the treatment of LHON in September 2015, an impairment of the Company's main intangible asset "Raxone" in 2012 in the amount of CHF 26.2 million was reversed.

## B. Selected financial information from the balance sheet

in CHF thousands	As of December 31,		2017	As of September 30, 2018
	2015	2016		
	<i>(audited)</i>			<i>(unaudited)</i>
Tangible assets .....	398	517	2,157	2,344
Intangible assets .....	29,559	26,549	23,560	27,443
Financial assets long-term .....	190	270	713	775
Restricted cash long-term .....	0	0	4,500	1,500
Deferred tax assets .....	3,061	1,106	1,242	1,245
<b>Non-current assets.....</b>	<b>33,208</b>	<b>28,442</b>	<b>32,172</b>	<b>33,307</b>
Prepaid expenses and accrued income .....	1,513	583	853	804
Inventories.....	3,441	7,676	10,147	9,319
Trade and other receivables.....	2,131	4,276	5,402	6,953
Financial assets short-term.....	0	0	13,011	5,735
Restricted cash short-term.....	0	0	3,000	3,000
Cash and cash equivalents.....	76,859	49,815	45,195	19,654
<b>Current assets.....</b>	<b>83,944</b>	<b>62,350</b>	<b>77,608</b>	<b>45,465</b>
<b>TOTAL ASSETS.....</b>	<b>117,152</b>	<b>90,792</b>	<b>109,780</b>	<b>78,772</b>
Share capital.....	6,263	6,280	6,289	6,528
Capital reserves and share premium.....	377,031	382,322	392,002	403,159
Retained earnings.....	(273,133)	(308,549)	(360,081)	(400,004)
Employee benefit reserve .....	(2,958)	(4,734)	(4,905)	(2,969)
Treasury shares .....	(177)	(172)	(335)	(1,145)
Other components of equity .....	(779)	(796)	(714)	(766)
<b>Total equity.....</b>	<b>106,247</b>	<b>74,351</b>	<b>32,256</b>	<b>4,803</b>
Convertible bonds .....	0	0	53,111	54,193
Derivate financial instruments .....	0	0	2,792	1,069
Pension liabilities .....	3,957	6,183	8,375	6,879
<b>Total non-current liabilities.....</b>	<b>3,957</b>	<b>6,183</b>	<b>64,278</b>	<b>62,141</b>
Trade and other payables .....	3,666	4,458	4,734	3,463
Accrued expenses.....	3,282	5,800	8,512	8,365
<b>Total current liabilities .....</b>	<b>6,948</b>	<b>10,258</b>	<b>13,246</b>	<b>11,828</b>
<b>TOTAL LIABILITIES .....</b>	<b>10,905</b>	<b>16,441</b>	<b>77,524</b>	<b>73,969</b>
<b>TOTAL EQUITY AND LIABILITIES .....</b>	<b>117,152</b>	<b>90,792</b>	<b>109,780</b>	<b>78,772</b>

### C. Selected financial information from the cash flow statement

in CHF thousands	For the financial year ended December 31,			For the nine months ended September 30,	
	2015	2016	2017	2017	2018
	<i>(audited)</i>			<i>(unaudited)</i>	
<b>Result before taxes .....</b>	<b>2,934</b>	<b>(33,194)</b>	<b>(51,275)</b>	<b>(34,228)</b>	<b>(39,543)</b>
Depreciation of tangible assets .....	85	168	257	175	455
Reversal of impairment on intangible assets .....	(26,157)	0	0	–	–
Amortization of intangible assets .....	1,037	3,096	3,125	2,338	2,360
Expenses for equity rights plans .....	2,040	4,683	9,687	5,518	5,241
Change in fair value of derivatives .....	0	0	(2,540)	(2,344)	(1,723)
Change in fair value of financial assets short-term .....	0	0	(96)	(117)	249
Other non-cash items (Polyphor clinical material) .....	0	0	0	0	290
Change in pension liabilities .....	(394)	450	2,021	584	440
Taxes paid .....	(46)	(266)	(392)	(293)	(383)
Changes in net working capital .....	(2,119)	(2,131)	315	(2,046)	311
Total financial result .....	239	67	821	71	2,376
<i>Interest received</i> <sup>(2)</sup> .....	2	5	5	0	1
<i>Interest paid</i> .....	(11)	(15)	(1,561)	(1,541)	(3,033)
<b>Cash flow from operating activities .....</b>	<b>(22,390)</b>	<b>(27,137)</b>	<b>(39,633)</b>	<b>(31,883)</b>	<b>(32,959)</b>
Investments in tangible assets .....	(350)	(289)	(1,261)	(439)	(1,271)
Investments in intangible assets .....	(165)	(86)	(136)	(104)	(33)
Investments in other financial assets short-term .....	0	0	(12,915)	(12,915)	0
Disposal of other financial assets short-term .....	0	0	0	0	7,027
Investments in other financial assets long-term .....	(104)	(84)	(427)	(426)	(70)
Change in restricted cash .....	0	0	(7,500)	(7,500)	3,000
<b>Cash flow from investing activities .....</b>	<b>(619)</b>	<b>(459)</b>	<b>(22,239)</b>	<b>(21,384)</b>	<b>8,653</b>
Capital increases from options exercised .....	2,127	385	34	0	0
Proceeds from options exercised .....	0	0	0	21	0
Proceeds from sale of treasury shares .....	0	418	9,372	7,437	1,894
Purchase of treasury shares .....	0	(172)	(9,567)	(7,626)	(3,049)
Proceeds from convertible bonds .....	0	0	57,269	57,269	0
Capital increase private placement .....	54,870	0	0	0	0
Capital increase .....	27,576	0	0	0	0
Cost of issuance of share capital .....	(1,943)	0	0	0	0
<b>Cash flow from financing activities .....</b>	<b>82,630</b>	<b>631</b>	<b>57,108</b>	<b>57,101</b>	<b>(1,155)</b>
Effects of exchange rate changes on cash and cash equivalents .....	(197)	(79)	144	94	(80)
<b>Net increase (decrease) in cash and cash equivalents .....</b>	<b>59,424</b>	<b>(27,044)</b>	<b>(4,620)</b>	<b>3,928</b>	<b>(25,541)</b>
Cash and cash equivalents at January 1 .....	17,435	76,859	49,815	49,815	45,195
<b>Cash and cash equivalents at December 31 or September 30, respectively .....</b>	<b>76,859</b>	<b>49,815</b>	<b>45,195</b>	<b>53,743</b>	<b>19,654</b>

## IX. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*The following discussion should be read in conjunction with the Audited Consolidated Financial Statements and the Unaudited Interim Condensed Consolidated Financial Statements as well as with "Selected Financial Data" and "Presentation of Financial and Other Information", included elsewhere in this Offering Memorandum.*

*In addition to historical data, the following section contains forward-looking statements, which are based on our management's assumptions regarding our future business performance. See "Forward-Looking Statements". A number of factors, including the risks described in the section titled "Risk Factors", may cause our actual results to differ materially from the results expected on the basis of these forward-looking statements.*

### A. Overview

We are a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. The portfolio comprises clinical stage treatments and a marketed treatment for neuro-ophthalmologic, neuromuscular and pulmonary diseases. We believe that we have developed significant expertise in the understanding of the underlying causes of these diseases and our goal is to become a market leader in the development and commercialization of products for their treatment.

Our lead compound is idebenone, an anti-oxidant agent that we believe can help improve production of energy by mitochondria in certain patients whose mitochondrial function is impaired by genetic defects. Idebenone is the active ingredient in Raxone<sup>®</sup>, our product marketed in the EU and Israel for the treatment of Leber's hereditary optic neuropathy ("LHON"), and a potential active ingredient or product candidate for the treatment of various mitochondrial, neuromuscular and ophthalmological diseases. Currently, we are focusing our development and commercialization efforts on our marketed product, Raxone<sup>®</sup> in LHON, and the late-stage indication of Raxone<sup>®</sup> in Duchenne muscular dystrophy ("DMD"). We are also exploring the early-stage product candidate omigapil for the treatment of congenital muscular dystrophy ("CMD").

In February 2018, we completed the first step in our strategy to in-license product candidates in neuro-ophthalmology, neuromuscular and pulmonary diseases by entering into a license agreement with Polyphor for POL6014. Under the agreement, we have obtained the worldwide, exclusive rights to develop and commercialize POL6014, an innovative macrocycle elastase inhibitor, and analogs, for an initial payment of CHF 6.5 million, payable in 238,924 Santhera shares at an agreed valuation of CHF 27.2053 per share and additional cash payments of up to CHF 121 million contingent on future development, regulatory and particularly sales milestones. In addition, Polyphor is entitled to tiered royalty payments from our future net sales of POL6014 and to certain milestone payments and royalties, provided that Santhera advances the development and market entry of POL6014 in other pulmonary diseases.

On November 20, 2018, we announced the signing of an agreement (the "**Option Agreement**") with Idorsia Pharmaceuticals Ltd, Allschwil, Switzerland ("**Idorsia**"), under which we have acquired an option to obtain from Idorsia an exclusive sub-license to commercialize the active ingredient vamorolone, a non-hormonal steroid modulator for the treatment of Duchenne Muscular Dystrophy ("**DMD**") developed by the U.S.-based company ReveraGen BioPharma, Inc. ("**ReveraGen**"). According to current research, vamorolone has a better tolerability profile than standard glucocorticoid steroids while maintaining its potential efficacy. Vamorolone is therefore considered a "dissociative" steroid. The therapeutic steroid effect of Vamorolone combined with an improved tolerability profile opens the possibility of a broad application in chronically treated inflammatory diseases, especially for diseases where children have to be treated with steroid-based anti-inflammatory drugs for a long time, e.g. patients with DMD. Vamorolone is currently in clinical development in young children with DMD and has the potential to replace current glucocorticoid steroids as a treatment for such patients. In addition, vamorolone also potentially offers a novel therapeutic approach

in other chronic inflammatory diseases. Vamorolone is being developed by ReveraGen, which holds patents; see “X. *The Company and Its Business—E. Additional information on our business—5. Market exclusivity and intellectual property*” beginning on page 90. ReveraGen has granted Idorsia a worldwide, unrestricted, exclusive option to license the product for commercialization under a collaborative agreement between the two parties entered into in April 2016. Idorsia has granted an option to sub-license vamorolone to us pursuant to the Option Agreement. Additionally, Idorsia will transfer know-how relevant for the development and commercialization of vamorolone as well as material and know-how for the drug production process to us. As a result, after the exercise of the option, we will become the sub-licensee of ReveraGen. Under the Option Agreement, we have acquired an option to obtain from Idorsia an exclusive sub-license to commercialize the active ingredient vamorolone for all indications and in all territories, except Japan and South Korea. We will be able to exercise this option at the latest when the data from the Phase IIB study in DMD patients are available, which is expected to be the case in 2020 according to the current development plan. Idorsia has the right under the Option Agreement, in its sole discretion, to grant us a sublicense for Japan and South Korea. As initial consideration for the acquisition of the option for the exclusive sublicense relating to ReveraGen’s vamorolone, we issued the 1,000,000 Idorsia Shares to Idorsia and have agreed to pay USD 20.0 million in cash. Both the issuance of the 1,000,000 Idorsia Shares to Idorsia and the payment of the USD 20.0 million in cash are non-refundable. The cash consideration of USD 20 million shall be paid at the earlier of (i) within three (3) business days of the resolution of the Company’s board of directors on the ascertainment and the execution of the capital increase regarding the Offered Shares, or (ii) within forty (40) days following November 20, 2018. If such payment is not made by Santhera within the applicable timeframe, Idorsia may terminate the Option Agreement with immediate effect and shall have the right to decide in its sole discretion on the enforcement of the USD 20.0 million payment. The cash payment amount is intended to compensate Idorsia for having already paid USD 15.0 million to ReveraGen to fund the ongoing Phase IIB trial in patients with DMD. The 1,000,000 Idorsia Shares came from authorized capital and were issued on November 21, 2018. Idorsia thus became our largest shareholder and holds 13.3% of the share capital (prior to the issuance of the Offered Shares). We intend to pay the USD 20.0 million cash component of the consideration from the net proceeds of the sale of the Offered Shares. Under the Option Agreement, Idorsia will be entitled to receive a cash payment from us of USD 30.0 million upon exercise of the option and commercial milestone payments of up to USD 80 million in the DMD indication and four one-time sales milestone payments of up to USD 130 million in aggregate. Regulatory milestone payments payable by the Company to Idorsia for three additional indications amount to up to USD 205 million in aggregate. Upon commercialization of vamorolone, the Company has committed to pay to Idorsia tiered royalties ranging from a single-digit to low double-digit percentage on the annual net sales of vamorolone.

We have incurred significant losses since our inception, including a net loss of CHF 8.0 million in 2014 (restated figure), CHF 21.2 million in 2015 (excluding the one-time effect of a reversal of an impairment), CHF 35.4 million in 2016, CHF 51.5 million in 2017 and CHF 39.9 million in the nine months ended September 30, 2018. As of September 30, 2018, we had an accumulated deficit of CHF 400.0 million. Our ability to generate product sales sufficient to achieve profitability will depend heavily on the successful development and commercialization of one or more of our product candidates.

## **B. Key factors affecting our results of operations**

The following key factors have contributed significantly to our results of operations during the periods under review and are reasonably likely to have a material effect on our results of operations in the future.

### **1. Net sales**

To date, our net sales have been limited to sales of Raxone<sup>®</sup> for the treatment of LHON. Given the low prevalence of LHON, we believe that the market for Raxone<sup>®</sup> in LHON is small and insufficient for us to become profitable. We, together with our distributors, currently sell Raxone<sup>®</sup> in LHON in more than 20 countries. In 2016, the first full year of commercialization of Raxone<sup>®</sup> in LHON, we generated net sales of Raxone<sup>®</sup> in LHON in the amount of CHF 19.0 million. In 2017, we generated net sales of Raxone<sup>®</sup> in LHON in the amount of CHF 22.9 million. In the nine months ended September 30, 2018, net sales of

Raxone<sup>®</sup> in LHON were CHF 23.6 million. We generate the majority of our net sales of Raxone<sup>®</sup> in LHON in France and Germany.

Our net sales generated from Raxone<sup>®</sup> in LHON have been strongly dependent on pricing and reimbursement decisions of third-party payers. For instance, the sales uptake of Raxone<sup>®</sup> in LHON in 2016 and 2017 was somewhat slower than originally expected due to the complex pricing and reimbursement processes in several EU markets, in some of which our requests for pricing and reimbursement were rejected or the pricing terms obtained are less favorable than as requested.

Our ability to increase our net sales in the future will largely depend on our being able to obtain marketing authorization and subsequently favorable pricing and reimbursement terms for Raxone<sup>®</sup> in DMD. In addition, we expect that pricing and reimbursement decisions regarding Raxone<sup>®</sup> in LHON will continue to have a considerable influence on our net sales in the future. Also, we expect the price of Raxone<sup>®</sup> and of any future products to erode substantially during any market exclusivity period, and we expect such price erosion to be accelerated after the end of any such market exclusivity.

## 2. Operating expenses

We expect our operating expenses to increase substantially compared to prior periods in connection with our ongoing activities, including our ongoing development and commercialization activities relating to Raxone<sup>®</sup>. Our operating expenses may vary substantially from period to period driven mainly by the progress of clinical trials, other development costs, post-authorization measures imposed on us by regulatory authorities and the ramp-up of commercialization activities if we obtain marketing authorization for Raxone<sup>®</sup> in DMD.

*Development expenses.* The majority of our development expenses consist of external development expenses (see note 11 to the Unaudited Interim Condensed Consolidated Financial Statements, note 20 to the 2017 Consolidated Financial Statements, note 20 to the 2016 Consolidated Financial Statements, and note 20 to the 2015 Consolidated Financial Statements) that we incur through our use of Clinical Research Organizations (“CROs”) and other third parties. The remainder of our development expenses primarily consist of employee expenses.

Our external and internal development expenses have primarily been driven by the status and progress of our clinical trials, particularly our ongoing phase III clinical trial for Raxone<sup>®</sup> in certain DMD patients with declining respiratory function who are receiving steroids (Phase III Delos) (see “—Our lead product candidate: Raxone<sup>®</sup> in DMD—Clinical development status” in “The Company and its Business” beginning on page 75 for more information), and by post-authorization measures relating to Raxone<sup>®</sup> in LHON (see “Market exclusivity, regulatory status and sales” in “The Company and its Business” beginning on page 73 for more information) during the period under review. We expect external and internal development costs to increase significantly for the foreseeable future as our development programs progress, including in connection with our efforts to reapply for an MAA for Raxone in DMD, which are expected for the first half of 2019, following our unsuccessful applications in 2017 if the European Commission grants us marketing authorization for Raxone<sup>®</sup> in DMD patients who are not receiving steroids, we expect that any required post-authorization measures will be a significant cost factor for us (see “—Our lead product candidate: Raxone<sup>®</sup> in DMD—Market exclusivity and regulatory status” in “The Company and its Business” beginning on page 76 for more information).

We do not believe that it is possible at this time to accurately project total program-specific expenses through and after commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including clinical trial outcomes, uncertainties around patient enrollment in clinical trials as well as future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time. Additionally, future commercial and regulatory factors beyond our control could impact our clinical development programs and plans.

*Reversal of impairment on intangible assets and inventories.* As a result of receiving marketing authorization for Raxone<sup>®</sup> in LHON in the EU in September 2015, we determined the recoverable amount of our

previously impaired intangible asset “Raxone”. This resulted in one-time income through the reversal of impairment of CHF 26.2 million on that asset, and the related intangible assets were transferred into assets available for use with a useful life of 10 years. At the same time, the impairment on some inventories was reversed in the amount of CHF 0.9 million. This reversal of impairment significantly affected our results of operations for 2015, but had no cash impact.

*Marketing and sales expenses.* We expect our marketing and sales expenses to increase substantially in the future if and when we are able to obtain marketing authorization for Raxone® in DMD in the EU. We also expect our marketing and sales expenses to increase to some extent in the near future in connection with the further rollout of Raxone® in LHON in the EU, and as a result of the ramp up of activities in the U.S., where our team currently manages our patient advocacy interactions, prepares for market entry in the U.S. and is the source of our U.S. regulatory and medical affairs expertise. All of these activities are associated with an increase in the headcount of our marketing and sales personnel and the related overhead.

*Employee expenses and benefits.* As of September 30, 2018, we had 119 employees (113.6 full-time equivalent). These figures are expected to increase significantly if and when we receive marketing authorization for Raxone® in DMD. Also, we expect that our ongoing expansion of operations relating to the commercialization of Raxone® in LHON, and the ramp up of activities in the U.S. will result in a moderate increase in employee expenses in the near term. We expect these developments to result in higher employee expenses in the future.

*License and collaboration agreements.* We have entered into various license and collaboration agreements that give third parties certain rights to royalty, milestone and/or success fees (see “—*License and collaboration agreements*” in “*The Company and its Business*” beginning on page 84 for more information):

- Takeda will be entitled to a percentage from future licensing and/or sales income generated by us from idebenone in DMD of up to EUR 7.0 million in aggregate. In addition, if we make use of our right to cross-reference Takeda’s idebenone data for regulatory use with respect to certain other indications, Takeda will be entitled to a percentage of future licensing and/or sales income generated by us from the relevant products of up to EUR 3.0 million in aggregate. In addition, Takeda will be entitled to receive a percentage of future income generated by us from idebenone in DMD or another indication that requires a cross-reference of Takeda’s idebenone data for regulatory use, but not more than EUR 1.0 million in aggregate.
- In February 2018, we completed the first step in our strategy to in-license product candidates in neuro-ophthalmology, neuromuscular and pulmonary diseases by entering into a license agreement with Polyphor for POL6014. Under the agreement, we have obtained the worldwide, exclusive rights to develop and commercialize POL6014, an innovative macrocycle elastase inhibitor, and analogs, for an initial payment of CHF 6.5 million, payable in 238,924 Santhera shares at an agreed valuation of CHF 27.2053 per share and additional cash payments of up to CHF 121 million contingent on future development, regulatory and particularly sales milestones. In addition, Polyphor is entitled to tiered royalty payments from our future net sales of POL6014 and to certain milestone payments and royalties, provided that Santhera advances the development and market entry of POL6014 in other pulmonary diseases.
- KU Leuven will be entitled to a success fee of up to EUR 0.4 million in aggregate if and when we obtain marketing authorization for Raxone® in DMD (or any other product covered by a patent or patent application that is the subject of our agreement with KU Leuven) in a major market, which includes the EU, the U.S., Canada, or Japan and certain countries within the EU. In addition, if we commercialize such product ourselves, KU Leuven would be entitled to 5% royalties on net sales generated by us from any such product. If we grant commercialization rights with regard to any such product to a third party, KU Leuven would be entitled to 15% of the consideration received by us from such third party.
- Novartis will be entitled to milestone payments upon the start of a clinical trial that is intended to result in an MAA or an NDA for omigapil in CMD, upon regulatory approval for such product in

a major market country, and after reaching certain sales levels of such product. Novartis would also be entitled to mid to high single-digit percent royalties on net sales generated from the sale of omigapil in CMD.

- On November 20, 2018 we have entered into an agreement with Idorsia Pharmaceuticals Ltd pursuant to which we will, subject to certain conditions, acquire the option to exclusively in-license, by way of sub-license, the first-in-class dissociative steroid vamorolone in all indications and all countries worldwide except Japan and South Korea. Vamorolone is currently in clinical development in young children with DMD and has the potential to replace current glucocorticoid steroids as a treatment for such patients. ReveraGen, the developer of vamorolone, has granted Idorsia a worldwide, unrestricted, exclusive option to license the product for commercialization under a collaborative agreement between the two parties entered into in April 2016. Under the agreement with Idorsia, we have acquired an option to obtain from Idorsia an exclusive sub-license to commercialize vamorolone for all indications and in all territories, except Japan and South Korea. As initial consideration for the acquisition of the option for the exclusive sub-license relating to vamorolone, we issued the 1,000,000 Idorsia Shares to Idorsia and have agreed to pay USD 20.0 million in cash, of which USD 15.0 million is intended to compensate Idorsia for having already paid USD 15.0 million to ReveraGen to fund the ongoing Phase IIb trial in patients with DMD. The 1,000,000 Idorsia Shares came from authorized capital and were issued on November 21, 2018. Idorsia thereby became our largest shareholder and holds 13.3% of the share capital (prior to the issuance of the Offered Shares). We intend to pay the USD 20.0 million cash component of the consideration from the net proceeds of the sale of the Offered Shares. Both the issuance of the 1,000,000 Idorsia Shares to Idorsia and the payment of the USD 20.0 million in cash are non-refundable. Under the Option Agreement, Idorsia will be entitled to receive a cash payment from us of USD 30.0 million upon exercise of the option and commercial milestone payments of up to USD 80 million in the DMD indication and four one-time sales milestone payments of up to USD 130 million in aggregate. Regulatory milestone payments payable by the Company to Idorsia for three additional indications amount to up to USD 205 million in aggregate. Upon commercialization of vamorolone, the Company has committed to pay to Idorsia tiered royalties ranging from a single-digit to low double-digit percentage on the annual net sales of vamorolone.

### 3. Financial expenses

*Convertible bonds.* On February 17, 2017, we issued an aggregate of CHF 60 million Senior Unsecured Convertible Bonds 2017-2022 (the “**Bonds**”). The Bonds carry interest at 5% *per annum* payable semi-annually on February 17 and August 17. As a result, our financial expenses (interest and amortization expenses) in the year ended December 31, 2017, increased significantly compared to historical levels.

### 4. Exchange rate fluctuations

While our reporting currency is Swiss francs, we generate the majority of our net sales in euro. The majority of our expenses from our business activities are currently denominated in Swiss francs, euros, and U.S. dollars. Since a majority of our net sales and a significant portion of our expenses are denominated in euros, we are naturally hedged to a certain extent. As of December 31, 2017, a 5% strengthening (weakening) of the euro against the Swiss franc, with all other variables held constant, would have had a positive (negative) effect on our result before taxes of CHF 0.4 million. In order to reduce our foreign exchange rate exposure, we may decide to enter into derivative currency contracts (forwards, options, structured derivatives) in the future to hedge against additional major foreign currency exchange rate fluctuations. Currently, we have no foreign exchange hedge contracts. In 2011, the Swiss franc substantially appreciated against most other currencies, prompting the Swiss National Bank to introduce a CHF 1.20 floor under the EUR/CHF exchange rate in September 2011. While the EUR/CHF exchange rate remained above CHF 1.20 following this measure, in January 2015, the Swiss National Bank removed this floor and the CHF substantially appreciated against most other currencies. Our results of operations have been and will continue to be affected

by currency exchange rates, primarily the EUR/CHF exchange rate and the USD/CHF exchange rate, and to some extent by the GBP/CHF exchange rate.

Translational effects of exchange rate fluctuations also arise because financial results of our subsidiaries are measured in the currency of the primary economic environment in which the subsidiary operates (its functional currency). The results of operations of our subsidiaries outside Switzerland are, therefore, measured in currencies other than Swiss francs and then translated into Swiss francs for presentation of our financial results in the consolidated financial statements of the Group. As currency exchange rates fluctuate, a subsidiary's financial results and thereby our financial results may be impacted as a result of such translation even though no real change in its results of operations has occurred.

### **C. Explanation of key line items in the consolidated income statement**

*Net sales* comprises the fair value of the sale of goods, net of value-added tax, rebates, discounts, returns and after eliminating intercompany sales. Revenue is recognized when title, risks and rewards of the products are transferred to purchasers.

*Cost of goods sold* comprises cost of goods sold, changes in inventories, amortization of intangibles, and cost of sales. Changes in inventories is used for booking movement in the inventory as per the end of the period. Cost of goods sold covers all expenses such as active pharmaceutical ingredients, production of finished drug products, packaging and amortization on intangible assets (and impairment). Cost of sales includes expenses for handling, transport (including transport insurance), logistics, product liability insurance, losses on receivables and royalties due on net sales.

*Amortization intangible asset* comprises the allocation of the amortizable amount of the intangible assets over their estimated useful life. In 2004, an intangible asset "Raxone" was defined. Such intangible asset became available for use in September 2015, after we received marketing authorization for Raxone® in LHON. Since then the intangible asset has been amortized.

*Development expenses* comprise primarily the costs associated with pre-clinical and clinical trials, depreciation and all related personnel expenses. They also comprise the cost of material used in pre-clinical and clinical testing, equity-based compensation, fees paid to consultants, cost for facilities, and depreciation of assets used.

*Reversal impairment on intangible assets and inventory* occurred in connection with the grant of marketing authorization for Raxone® in LHON in 2015, as a result of which the Company reversed the impairment on intangible assets and inventories that had been made as per the end of 2012.

*Marketing and sales* mainly comprises marketing- and sales-related personnel expenses, IT, facility-related expenses as well as depreciation and amortization.

*General and administrative* mainly comprises headquarter-related personnel expenses, IT and consulting services expenses, facility-related expenses as well as depreciation and amortization.

*Financial income* comprises foreign exchange gains (realized and unrealized), interest income, financial instruments income, dividend income and income from financial assets.

*Financial expenses* comprise foreign exchange losses (realized and unrealized) and interest expenses, primarily on the Bonds that we issued in February 2017.

*Income taxes* comprise current income tax expenses or income, deferred income tax expenses or income, and other tax expenses or income.

## D. Results of operations

The following table sets out certain information from our consolidated income statements for the years ended December 31, 2015, 2016 and 2017 and for the nine months ended September 30, 2017 and 2018:

in CHF thousands	For the financial year ended December 31,			For the nine months ended September 30,	
	2015	2016	2017	2017	2018
	<i>(audited)</i>			<i>(unaudited)</i>	
<b>Net sales</b> .....	<b>4,321</b>	<b>19,033</b>	<b>22,943</b>	<b>16,347</b>	<b>23,634</b>
Cost of goods sold.....	(1,371)	(3,883)	(4,104)	(3,028)	(3,606)
<i>Of which amortization intangible asset</i> .....	<i>(1,013)</i>	<i>(3,039)</i>	<i>(3,039)</i>	<i>(2,279)</i>	<i>(2,279)</i>
Other operating income.....	188	361	270	243	1
Development.....	16,651	(17,675)	(26,561)	(18,168)	(27,098)
<i>Of which development expenses</i> .....	<i>(10,453)</i>	<i>(17,675)</i>	<i>(26,561)</i>	<i>(18,168)</i>	<i>(27,098)</i>
<i>Of which reversal impairment on intangible assets and inventory</i> .....	<i>27,104<sup>(1)</sup></i>	<i>0</i>	<i>0</i>	<i>0</i>	<i>0</i>
Marketing and sales .....	(8,356)	(21,051)	(28,522)	(19,909)	(18,637)
General and administrative .....	(8,244)	(9,805)	(14,416)	(9,573)	(11,292)
Other operating expenses .....	(16)	(107)	(64)	(68)	(169)
<b>Operating expenses</b> .....	<b>35</b>	<b>(48,638)</b>	<b>(69,563)</b>	<b>(47,718)</b>	<b>(57,196)</b>
<b>Operating result</b> .....	<b>3,173</b>	<b>(33,127)</b>	<b>(50,454)</b>	<b>(34,156)</b>	<b>(37,167)</b>
Financial income .....	416	928	4,134	3,533	2,856
Financial expenses .....	(655)	(995)	(4,955)	(3,605)	(5,232)
<b>Result before taxes</b> .....	<b>2,934</b>	<b>(33,194)</b>	<b>(51,275)</b>	<b>(34,228)</b>	<b>(39,543)</b>
Income taxes .....	3,015	(2,221)	(257)	895	(380)
<b>Net result</b> .....	<b>5,949</b>	<b>(35,415)</b>	<b>(51,532)</b>	<b>(33,333)</b>	<b>(39,923)</b>
Items never to be reclassified to net income in subsequent periods					
<i>Actuarial gains (losses) on defined benefit plans</i> .....	<i>(1,671)</i>	<i>(1,776)</i>	<i>(171)</i>	<i>137</i>	<i>1,936</i>
Items to be reclassified to net income in subsequent periods					
<i>Currency translation differences</i> .....	<i>(16)</i>	<i>(18)</i>	<i>82</i>	<i>60</i>	<i>(52)</i>
<b>Other comprehensive result</b> .....	<b>(1,687)</b>	<b>(1,794)</b>	<b>(89)</b>	<b>197</b>	<b>1,884</b>
<b>Total comprehensive result</b> .....	<b>4,262</b>	<b>(37,209)</b>	<b>(51,621)</b>	<b>(33,136)</b>	<b>(38,039)</b>

(1) As a result of receiving marketing authorization in the EU for Raxone® for the treatment of LHON in September 2015, an impairment of the Company's main intangible asset "Raxone" made in 2012 in the amount of CHF 26.2 million was reversed.

1. **Nine months ended September 30, 2018 compared to nine months ended September 30, 2017**

	For the nine months ended September 30,				Change	
	2017 (Base)	% of Net Sales	2018	% of Net Sales	in CHF	in %
in CHF thousands, except percentages						
<b>Consolidated Income Statement</b>						
<b>Net sales</b> .....	<b>16,347</b>	<b>100.0%</b>	<b>23,634</b>	<b>100.0%</b>	<b>7,287</b>	<b>44.6%</b>
Cost of goods sold.....	(3,028)	(18.5)%	(3,606)	(15.3)%	(578)	19.1%
<i>Of which amortization intangible as-</i>						
<i>set</i> .....	(2,279)	(13.9)%	(2,279)	(9.6)%	0	0.0%
Other operating income.....	243	1.5%	1	0.0%	(242)	(99.6)%
Development .....	(18,168)	(111.1)%	(27,098)	(114.7)%	(8,930)	49.2%
Marketing and sales .....	(19,909)	(121.8)%	(18,637)	(78.9)%	1,272	(6.4)%
General and administrative .....	(9,573)	(58.6)%	(11,292)	(47.8)%	(1,719)	18.0%
Other operating expenses .....	(68)	(0.4)%	(169)	(0.7)%	(101)	148.5%
<b>Operating expenses</b> .....	<b>(47,718)</b>	<b>(291.9)%</b>	<b>(57,196)</b>	<b>(242.0)%</b>	<b>(9,478)</b>	<b>19.9%</b>
<b>Operating result</b> .....	<b>(34,156)</b>	<b>(208.9)%</b>	<b>(37,167)</b>	<b>(157.3)%</b>	<b>(3,011)</b>	<b>8.8%</b>
Financial income .....	3,533	21.6%	2,856	12.1%	(677)	(19.2)%
Financial expenses .....	(3,605)	(22.1)%	(5,232)	(22.1)%	1,627	45.1%
<b>Result before taxes</b> .....	<b>(34,228)</b>	<b>(209.4)%</b>	<b>(39,543)</b>	<b>(167.3)%</b>	<b>(5,315)</b>	<b>15.5%</b>
Income taxes .....	895	5.5%	(380)	(1.6)%	(1,275)	(142.5)%
<b>Net result</b> .....	<b>(33,333)</b>	<b>(203.9)%</b>	<b>(39,923)</b>	<b>(168.9)%</b>	<b>(6,590)</b>	<b>19.8%</b>

a. **Net sales**

Net sales were CHF 23.6 million in the nine months ended September 30, 2018, an increase of CHF 7.3 million, or 44.6%, from CHF 16.3 million in the nine months ended September 30, 2017. The increase was primarily due to the continued rollout of Raxone® in LHON, which we had launched for sale in the EU in October 2015 and which was sold in more countries and to more patients during the nine months ended September 30, 2018, compared to the nine months ended September 30, 2017. During both periods, we generated the majority of our sales of Raxone® in LHON in France and Germany.

b. **Cost of goods sold**

The table below sets forth our cost of goods sold for the nine months ended September 30, 2018 compared to the nine months ended September 30, 2017:

	For the nine months ended September 30,				Change	
	2017 (Base)	% of Net Sales	2018	% of Net Sales	in CHF	in %
in CHF thousands, except percentages						
Cost of goods sold.....	(3,028)	(18.5)%	(3,606)	(15.3)%	(578)	19.1%
<i>Of which amortization intangible as-</i>						
<i>set</i> .....	(2,279)	(13.9)%	(2,279)	(9.6)%	0	0.0%

Cost of goods sold was CHF 3.6 million in the nine months ended September 30, 2018, an increase of CHF 0.6 million, or 19.1%, from CHF 3.0 million in the nine months ended September 30, 2017. The increase was primarily due to an increased sales volume of Raxone® in LHON and additional expenses in connection with the implementation of Directive 2011/62/EC (the “**Falsified Medicines Directive**”) in EU member states.

**c. Development**

Development expenses were CHF 27.1 million in the nine months ended September 30, 2018, an increase of CHF 8.9 million, or 49.2%, from CHF 18.2 million in the nine months ended September 30, 2017. The increase was primarily due to increased expenses for clinical trials (including expenses associated with our ongoing phase III clinical trial (SIDEROS) of Raxone® in certain DMD patients and with POL6014 in patients with CF), for regulatory matters and medical affairs, as well as increased personnel expenses primarily as a result of an increase in headcount, an increase in (non-cash-relevant) expenses for equity rights plans, and an expansion of pension benefits.

**d. Marketing and sales**

Marketing and sales expenses were CHF 18.6 million in the nine months ended September 30, 2018, a decrease of CHF 1.3 million, or 6.4%, from CHF 19.9 million in the nine months ended September 30, 2017. The decrease was primarily due to preparatory expenses in the nine months ended September 30, 2017 in connection with the preparation for launch in case of an expected positive opinion of the CHMP for the Marketing Authorization Application for DMD in the EU.

**e. General and administrative expenses**

General and administrative expenses were CHF 11.3 million in the nine months ended September 30, 2018, an increase of CHF 1.7 million, or 18.0%, from CHF 9.6 million in the nine months ended September 30, 2017. The increase was primarily due to increased personnel expenses primarily as a result of an increase in headcount and an increase in (non-cash-relevant) expenses for equity rights plans, and an expansion of pension benefits.

**f. Operating expenses**

As a result of the factors described above, operating expenses were CHF 57.2 million in the nine months ended September 30, 2018, an increase of CHF 9.5 million, or 19.9%, from CHF 47.7 million in the nine months ended September 30, 2017.

**g. Operating result**

As a result of the factors described above, the operating result was a negative CHF 37.2 million in the nine months ended September 30, 2018, an increase of CHF 3.0 million, or 8.8%, from a negative CHF 34.2 million in the nine months ended September 30, 2017.

**h. Financial income**

Financial income was CHF 2.9 million in the nine months ended September 30, 2018, a decrease of CHF 0.7, or 19.2%, from CHF 3.5 million in the nine months ended September 30, 2017. The decrease was primarily due to lower income in connection with the valuation of the derivatives relating to the the Company's CHF 60 million Senior Unsecured Convertible Bonds 2017-2022.

**i. Financial expenses**

Financial expenses were CHF 5.2 million in the nine months ended September 30, 2018, an increase of CHF 1.6 million, or 45.1%, from CHF 3.6 million in the nine months ended September 30, 2017. The increase was primarily due to higher interest expenses on our Bonds issued in February 2017 and, to a lesser degree, higher foreign exchange fluctuations and related valuation losses.

**j. Result before taxes**

As a result of the factors described above, the result before taxes was a negative CHF 39.5 million in the nine months ended September 30, 2018, an increase of CHF 5.3 million, or 15.5%, from a negative CHF 34.2 million in the nine months ended September 30, 2017.

**k. Income tax**

Income tax expenses were CHF 0.4 million in the nine months ended September 30, 2018, an increase of CHF 1.3 million, or 142.5%, from an income tax benefit of CHF 0.9 million in the nine months ended September 30, 2017.

**l. Net result**

As a result of the factors described above, the net result was a negative CHF 39.9 million in the nine months ended September 30, 2018, an increase of CHF 6.6 million, or 19.8%, from a negative CHF 33.3 million in the nine months ended September 30, 2017.

**2. Financial year ended December 31, 2017 as compared to the financial year ended December 31, 2016**

	For the financial year ended December 31,				Change	
	2016 (Base Year)	% of Net Sales	2017	% of Net Sales	in CHF	in %
in CHF thousands, except percentages						
<b>Consolidated Income Statement</b>						
<b>Net sales .....</b>	<b>19,033</b>	<b>100.0%</b>	<b>22,943</b>	<b>100.0%</b>	<b>3,910</b>	<b>20.5%</b>
Cost of goods sold.....	(3,883)	(20.4)%	(4,104)	(17.9)%	(221)	5.7%
<i>Of which amortization intangible as-</i> <i>set.....</i>	<i>(3,039)</i>	<i>(16.0)%</i>	<i>(3,039)</i>	<i>(13.2)%</i>	<i>0</i>	<i>0.0%</i>
Other operating income.....	361	1.9%	270	1.2%	(91)	(25.2)%
Development .....	(17,675)	(92.9)%	(26,561)	(115.8)%	(8,886)	50.3%
Marketing and sales .....	(21,051)	(110.6)%	(28,522)	(124.3)%	(7,471)	35.5%
General and administrative .....	(9,805)	(51.5)%	(14,416)	(62.8)%	(4,611)	47.0%
Other operating expenses .....	(107)	(0.6)%	(64)	(0.3)%	43	(40.2)%
<b>Operating expenses .....</b>	<b>(48,638)</b>	<b>(255.5)%</b>	<b>(69,563)</b>	<b>(303.2)%</b>	<b>(20,925)</b>	<b>43.0%</b>
<b>Operating result .....</b>	<b>(33,127)</b>	<b>(174.1)%</b>	<b>(50,454)</b>	<b>(219.9)%</b>	<b>(17,327)</b>	<b>52.3%</b>
Financial income .....	928	4.9%	4,134	18.0%	3,206	345.5%
Financial expenses .....	(995)	(5.2)%	(4,955)	(21.6)%	(3,960)	398.0%
<b>Result before taxes .....</b>	<b>(33,194)</b>	<b>(174.4)%</b>	<b>(51,275)</b>	<b>(223.5)%</b>	<b>(18,081)</b>	<b>54.5%</b>
Income taxes .....	(2,221)	(11.7)%	(257)	(1.1)%	1,964	(88.4)%
<b>Net result.....</b>	<b>(35,415)</b>	<b>(186.1)%</b>	<b>(51,532)</b>	<b>(224.6)%</b>	<b>(16,117)</b>	<b>45.5%</b>

**a. Net sales**

Net sales were CHF 22.9 million in the financial year ended December 31, 2017, an increase of CHF 3.9 million, or 20.5%, from CHF 19.0 million in the financial year ended December 31, 2016. The increase was primarily due to the continued rollout of Raxone® in LHON, which we had launched for sale in the EU in October 2015 and which was sold in more countries and to more patients during the financial year ended December 31, 2017, compared to the financial year ended December 31, 2016. During both periods, we generated the majority of our sales of Raxone® in LHON in France and Germany.

**b. Cost of goods sold**

The table below sets forth our cost of goods sold for the financial year ended December 31, 2017, as compared to the financial year ended December 31, 2016:

	For the financial year ended December 31,				Change	
	2016 (Base Year)	% of total	2017	% of total	in CHF	in %
in CHF thousands, except percentages						
Cost of goods sold.....	(3,883)	100.0%	(4,104)	100.0%	(221)	5.7%
<i>Of which amortization intangible as- set.....</i>	<i>(3,039)</i>	<i>78.3%</i>	<i>(3,039)</i>	<i>74.0%</i>	<i>0</i>	<i>0.0%</i>

Cost of goods sold was CHF 4.1 million in the financial year ended December 31, 2017, an increase of CHF 0.2 million, or 5.7%, from CHF 3.9 million in the financial year ended December 31, 2016. The increase was primarily due to an increased sales volume of Raxone® in LHON.

**c. Development**

Development expenses were CHF 26.6 million in the financial year ended December 31, 2017, an increase of CHF 8.9 million, or 50.3%, from CHF 17.7 million in the financial year ended December 31, 2016. The increase was primarily due to increased expenses for clinical trials (including expenses associated with our ongoing phase III clinical trial (SIDEROS) of Raxone® in certain DMD patients), for regulatory matters and medical affairs, as well as increased personnel expenses primarily as a result of an increase in headcount, an increase in (non-cash-relevant) expenses for equity rights plans, and an expansion of pension benefits.

**d. Marketing and sales**

Marketing and sales expenses were CHF 28.5 million in the financial year ended December 31, 2017, an increase of CHF 7.5 million, or 35.5%, from CHF 21.1 million in the financial year ended December 31, 2016. The increase was primarily due to additional marketing activities regarding Raxone in LHON® in Europe and to prepare for a timely market entry of Raxone® in DMD in the EU in anticipation of marketing authorization, which we expected but failed to receive in early 2018, the ramp up of activities in the U.S., where our team currently prepares for market entry in the U.S., among other things, as well as increased personnel expenses primarily as a result of an increase in headcount and an increase in (non-cash-relevant) expenses for equity rights plans.

**e. General and administrative expenses**

General and administrative expenses were CHF 14.4 million in the financial year ended December 31, 2017, an increase of CHF 4.6 million, or 47.0%, from CHF 9.8 million in the financial year ended December 31, 2016. The increase was primarily due to increased personnel expenses primarily as a result of an increase in headcount and an increase in (non-cash-relevant) expenses for equity rights plans, and an expansion of pension benefits.

**f. Operating expenses**

As a result of the factors described above, operating expenses were CHF 69.6 million in the financial year ended December 31, 2017, an increase of CHF 20.9 million, or 43.0%, from CHF 48.6 million in the financial year ended December 31, 2016.

**g. Operating result**

As a result of the factors described above, the operating result was a negative CHF 50.5 million in the financial year ended December 31, 2017, an increase of CHF 17.3 million, or 52.3%, from a negative CHF 33.1 million in the financial year ended December 31, 2016.

**h. Financial income**

Financial income was CHF 4.1 million in the financial year ended December 31, 2017, an increase of CHF 3.2 million, or 345.5%, from CHF 0.9 million in the financial year ended December 31, 2016. The increase was primarily due to a change in fair value of the financial derivatives (conversion right, reset mechanism and early redemption option) embedded in our Bonds issued in February 2017 and, to a lesser degree, higher foreign exchange fluctuations on additional foreign currencies and related valuation gains.

**i. Financial expenses**

Financial expenses were CHF 5.0 million in the financial year ended December 31, 2017, an increase of CHF 4.0 million, or 398.0%, from CHF 1.0 million in the financial year ended December 31, 2016. The increase was primarily due to interest expenses on our Bonds issued in February 2017 and, to a lesser degree, higher foreign exchange fluctuations and related valuation losses.

**j. Result before taxes**

As a result of the factors described above, the result before taxes was a negative CHF 51.3 million in the financial year ended December 31, 2017, an increase of CHF 18.1 million, or 54.5%, from a negative CHF 33.2 million in the financial year ended December 31, 2016.

**k. Income tax**

Income tax expenses were CHF 0.3 million in the financial year ended December 31, 2017, a decrease of CHF 2.0 million, or 88.4%, from CHF 2.2 million in the financial year ended December 31, 2016. The decrease was primarily due to the fact that the deferred tax expense resulting from inventory movements from December 31, 2015, to December 31, 2016, was a one-time effect in the financial year ended December 31, 2016.

**l. Net result**

As a result of the factors described above, the net result was a negative CHF 51.5 million in the financial year ended December 31, 2017, an increase of CHF 16.1 million, or 45.5%, from a negative CHF 35.4 million in the financial year ended December 31, 2016.

**3. Financial year ended December 31, 2016 as compared to the financial year ended December 31, 2015**

	For the financial year ended December 31,				Change	
	2015	% of	2016	% of	in CHF	in %
	(Base	Net Sales		Net Sales		
	Year)					
in CHF thousands, except percentages						
<b>Consolidated Income Statement</b>						
Net sales .....	4,321	100.0%	19,033	100.0%	14,712	340.5%
Cost of goods sold.....	(1,371)	(31.7)%	(3,883)	(20.4)%	(2,512)	183.2%
<i>Of which amortization intangible as-</i>						
<i>set .....</i>	(1,013)	(23.4)%	(3,039)	(16.0)%	(2,026)	200.0%
Other operating income.....	188	4.4%	361	1.9%	173	92.0%
Development .....	16,651	385.4%	(17,675)	(92.9)%	(34,326)	n.m.
<i>Of which development expenses.....</i>	(10,453)	(241.9)%	(17,675)	(92.9)%	(7,222)	69.1%

<i>Of which reversal impairment on intangible assets and inventory.....</i>	27,104	627.3%	0	0.0%	(27,104)	(100.0)%
Marketing and sales .....	(8,356)	(193.4)%	(21,051)	(110.6)%	(12,695)	151.9%
General and administrative .....	(8,244)	(190.8)%	(9,805)	(51.5)%	(1,561)	18.9%
Other operating expenses .....	(16)	(0.4)%	(107)	(0.6)%	(91)	568.8%
<b>Operating expenses .....</b>	<b>35</b>	<b>0.8%</b>	<b>(48,638)</b>	<b>(255.5)%</b>	<b>(48,673)</b>	<b>n.m.</b>
<b>Operating result .....</b>	<b>3,173</b>	<b>73.4%</b>	<b>(33,127)</b>	<b>(174.1)%</b>	<b>(36,300)</b>	<b>n.m.</b>
Financial income .....	416	9.6%	928	4.9%	512	123.1%
Financial expenses .....	(655)	(15.2)%	(995)	(5.2)%	(340)	51.9%
<b>Result before taxes .....</b>	<b>2,934</b>	<b>67.9%</b>	<b>(33,194)</b>	<b>(174.4)%</b>	<b>(36,128)</b>	<b>n.m.</b>
Income taxes .....	3,015	69.8%	(2,221)	(11.7)%	(5,236)	n.m.
<b>Net result.....</b>	<b>5,949</b>	<b>137.7%</b>	<b>(35,415)</b>	<b>(186.1)%</b>	<b>(41,364)</b>	<b>n.m.</b>

**a. Net sales**

Net sales were CHF 19.0 million in the financial year ended December 31, 2016, an increase of CHF 14.7 million, or 340.5%, from CHF 4.3 million in the financial year ended December 31, 2015. The increase was primarily due to the fact that Raxone® in LHON was sold during the full year of 2016 after its launch for sale in the EU in October 2015 (in the first three quarters of 2015, it had been sold under special programs such as a temporary authorization in France).

**b. Cost of goods sold**

The table below sets forth our cost of goods sold for the financial year ended December 31, 2016, as compared to the financial year ended December 31, 2015:

	For the financial year ended December 31,				Change	
	2015 (Base Year)	% of total	2016	% of total	in CHF	in %
in CHF thousands, except percentages						
Cost of goods sold.....	(1,371)	100.0%	(3,883)	100.0%	(2,512)	183.2%
<i>Of which amortization intangible as- set.....</i>	<i>(1,013)</i>	<i>73.9%</i>	<i>(3,039)</i>	<i>78.3%</i>	<i>(2,026)</i>	<i>200.0%</i>

Cost of goods sold was CHF 3.9 million in the financial year ended December 31, 2016, an increase of CHF 2.5 million, or 183.2%, from CHF 1.4 million in the financial year ended December 31, 2015. The increase was primarily due to increased net sales, as we were able to increase sales of Raxone® in LHON after having received marketing authorization in the EU in October 2015.

**c. Development**

The table below sets forth our development expenses for the financial year ended December 31, 2016, as compared to the financial year ended December 31, 2015:

	For the financial year ended December 31,				Change	
	2015 (Base Year)	% of total	2016	% of total	in CHF	in %
in CHF thousands, except percentages						
Development.....	16,651	100.0%	(17,675)	100.0%	(34,326)	n.m.
<i>Of which development expenses.....</i>	<i>(10,453)</i>	<i>(62.8)%</i>	<i>(17,675)</i>	<i>100.0%</i>	<i>(7,222)</i>	<i>69.1%</i>
<i>Of which reversal impairment on intangible assets and inventory.....</i>	<i>27,104</i>	<i>162.8%</i>	<i>0</i>	<i>0.0%</i>	<i>(27,104)</i>	<i>(100.0)%</i>

Development expenses were CHF 17.7 million in the financial year ended December 31, 2016, a net change of CHF 34.3 million from income of CHF 16.7 million in the financial year ended December 31, 2015. Development expenses in 2015 were significantly impacted by the one-time effect of a reversal of an impairment on intangible assets and inventories: as a result of receiving marketing authorization for Raxone® in LHON in the EU in September 2015, we determined the recoverable amount of our previously impaired intangible asset “Raxone”. This resulted in a reversal of impairment of CHF 26.2 million on that asset. At the same time, we reversed the impairment on some inventories in the amount of CHF 0.9 million. Excluding the effects related to such impairment reversals, our development expenses increased from CHF 10.5 million to CHF 17.7 million. The higher development expenses were primarily attributable to expenses associated with post-authorization measures relating to Raxone® in LHON, increased expenses associated with our ongoing phase III clinical trial (SIDEROS) of Raxone® in certain DMD patients with declining respiratory function who are receiving steroids, as well as expenses associated with regulatory filings.

**d. Marketing and sales**

Marketing and sales expenses were CHF 21.1 million in the financial year ended December 31, 2016, an increase of CHF 12.7 million, or 151.9%, from CHF 8.4 million in the financial year ended December 31, 2015. The increase was primarily due to the commercial roll-out of Raxone® in LHON in the EU, preparations for market entry in the second indication DMD and the build-up of the operations in the U.S.

**e. General and administrative expenses**

General and administrative expenses were CHF 9.8 million in the financial year ended December 31, 2016, an increase of CHF 1.6 million, or 18.9%, from CHF 8.2 million in the financial year ended December 31, 2015. The increase was primarily due to increased personnel expenses and consultancy fees, which were mainly due to an increase in headcount for supporting the expanding development, marketing and sales activities.

**f. Operating expenses**

As a result of the factors described above, operating expenses were CHF 48.7 million in the financial year ended December 31, 2016, a net change of CHF 48.7 million from income of CHF 0.0 million in the financial year ended December 31, 2015.

**g. Operating result**

As a result of the factors described above, the operating result was a negative CHF 33.1 million in the financial year ended December 31, 2016, a net change of CHF 36.3 million from a positive operating result of CHF 3.2 million in the financial year ended December 31, 2015.

**h. Financial income**

Financial income was CHF 0.9 million in the financial year ended December 31, 2016, an increase of CHF 0.5 million, or 123.1%, from CHF 0.4 million in the financial year ended December 31, 2015. The increase was primarily due to foreign exchange fluctuations on higher foreign currency amounts and related valuation gains.

**i. Financial expenses**

Financial expenses were CHF 1.0 million in the financial year ended December 31, 2016, an increase of CHF 0.3 million, or 51.9%, from CHF 0.7 million in the financial year ended December 31, 2015. The increase was primarily due to foreign exchange fluctuations on higher foreign currency amounts and related valuation losses.

**j. Result before taxes**

As a result of the factors described above, the result before taxes was a negative CHF 33.2 million in the financial year ended December 31, 2016, a net change of CHF 36.1 million from the positive result before taxes of CHF 2.9 million in the financial year ended December 31, 2015.

**k. Income taxes**

Income tax expenses were CHF 2.2 million in the financial year ended December 31, 2016, a net change of CHF 5.2 million from income of CHF 3.0 million in the financial year ended December 31, 2015. The net change was primarily due to movement on deferred tax assets. Deferred tax assets on inventory were initially recognized in the financial year ended December 31, 2015, which led to an income of CHF 3.1 million. Inventory movements from December 31, 2015, to December 31, 2016, led to a reduction of deferred tax assets and hence a deferred tax expense of CHF 2.0 million.

**l. Net result**

As a result of the factors described above, the net result was a negative CHF 35.4 million in the financial year ended December 31, 2016, a net change of CHF 41.4 million from a positive net result of CHF 5.9 million in the financial year ended December 31, 2015.

**E. Funding, Liquidity and Capital Resources**

**1. General**

Since our inception, we have incurred significant operating losses (with the exception of 2015, when we recorded a positive operating result as a result of the one-time effect of a reversal of an impairment in the aggregate amount of CHF 27.1 million, of which CHF 26.2 million related to intangible assets and CHF 0.9 million related to inventories, that had no cash impact). We have generated limited sales to date from the sale of our only marketed product, Raxone® in LHON. We rely on cash flow from operating activities, available cash and cash equivalents, as well as the raising of equity capital and equity-linked debt, to finance our operations and business expansion. Since January 1, 2014, we have raised equity capital through several capital increases. In 2014, we raised aggregate gross proceeds of CHF 15.7 million from the sale of shares for financing purposes. We received an additional CHF 3.2 million in cash in connection with the exercise of share options in 2014. In August 2015, we raised gross proceeds of CHF 27.7 million in a private placement. In December 2015, we raised gross proceeds of CHF 54.9 million in a further private placement. We received an additional CHF 2.1 million in cash in connection with the exercise of share options in 2015, and CHF 0.4 million in 2016. In February 2017, we raised aggregate gross proceeds of CHF 60 million from the issuance of the Bonds. Out of the net proceeds of the Bonds, an amount corresponding to the interest payable on the Bonds for the first three years of their term was put into escrow to be used for interest payments and is not available for other purposes.

As of September 30, 2018, we had cash and cash equivalents of CHF 19.7 million, excluding the restricted cash we placed in escrow for interest payments during the first three years of the term of our Bonds. Based on our current cash projections, we believe that, even upon successful consummation of the Offering and assuming that we raise the targeted gross proceeds of approximately CHF 50 million, our cash and cash equivalents will be sufficient to fund our anticipated capital expenditures, operational expenditures and working capital requirements only for the immediately foreseeable future in 2019. Therefore we will likely need to raise further equity and/or debt financing in the future, which could be as early as during 2019. We cannot predict whether any additional financing will be available at all or available on commercially acceptable terms when needed. We have based these estimates on assumptions that may prove to be wrong, and we could be required to use our capital resources sooner than expected or for purposes other than those that we currently expect. If we are unable to raise the targeted proceeds in this Offering, we will need to raise additional funds (equity and/or debt financing) in the immediate short term in order to continue our

operations as planned. Without such funds, there will be material uncertainty as to whether we will be able to continue as a going concern for another twelve months.

Our liquidity requirements primarily relate to funding our development and commercialization activities, to fees and expenses, including upfront fees and any future milestone payments and royalties, for in-licensed products and product candidates, as well as our operating and financing expenses.

The most significant components of our net working capital are cash and cash equivalents, short-term financial assets and restricted cash, trade and other receivables and inventories as well as accrued expenses and trade and other payables.

## 2. Cash flows

The following table sets out certain cash flow information for the years ended December 31, 2015, 2016 and 2017 and for the nine months ended September 30, 2017 and 2018:

in CHF thousands	For the financial year ended December 31,			For the nine months ended September 30,	
	2015	2016	2017	2017	2018
	<i>(audited)</i>			<i>(unaudited)</i>	
<b>Result before taxes</b> .....	<b>2,934</b>	<b>(33,194)</b>	<b>(51,275)</b>	<b>(34,228)</b>	<b>(39,543)</b>
Depreciation of tangible assets.....	85	168	257	175	455
Reversal of impairment on intangible assets .....	(26,157)	0	0	–	–
Amortization of intangible assets .....	1,037	3,096	3,125	2,338	2,360
Expenses for equity rights plans.....	2,040	4,683	9,687	5,518	5,241
Change in fair value of derivatives.....	0	0	(2,540)	(2,344)	(1,723)
Change in fair value of financial assets short-term .....	0	0	(96)	(117)	249
Other non-cash items (Polyphor clinical material)	0	0	0	0	290
Change in pension liabilities .....	(394)	450	2,021	584	440
Taxes paid .....	(46)	(266)	(392)	(293)	(383)
Changes in net working capital .....	(2,119)	(2,131)	315	(2,046)	311
Total financial result .....	239	67	821	71	2,376
Interest received <sup>(2)</sup> .....	2	5	5	0	1
Interest paid .....	(11)	(15)	(1,561)	(1,541)	(3,033)
<b>Cash flow from operating activities</b> .....	<b>(22,390)</b>	<b>(27,137)</b>	<b>(39,633)</b>	<b>(31,883)</b>	<b>(32,959)</b>
Investments in tangible assets .....	(350)	(289)	(1,261)	(439)	(1,271)
Investments in intangible assets .....	(165)	(86)	(136)	(104)	(33)
Investments in other financial assets short-term.....	0	0	(12,915)	(12,915)	0
Disposal of other financial assets short-term.....	0	0	0	0	7,027
Investments in other financial assets long-term .....	(104)	(84)	(427)	(426)	(70)
Change in restricted cash .....	0	0	(7,500)	(7,500)	3,000
<b>Cash flow from investing activities</b> .....	<b>(619)</b>	<b>(459)</b>	<b>(22,239)</b>	<b>(21,384)</b>	<b>8,653</b>
Capital increases from options exercised .....	2,127	385	34	0	0
Proceeds from options exercised.....	0	0	0	21	0
Proceeds from sale of treasury shares .....	0	418	9,372	7,437	1,894
Purchase of treasury shares .....	0	(172)	(9,567)	(7,626)	(3,049)
Proceeds from convertible bonds .....	0	0	57,269	57,269	0
Capital increase private placement.....	54,870	0	0	0	0
Capital increase .....	27,576	0	0	0	0
Cost of issuance of share capital .....	(1,943)	0	0	0	0
<b>Cash flow from financing activities</b> .....	<b>82,630</b>	<b>631</b>	<b>57,108</b>	<b>57,101</b>	<b>(1,155)</b>
Effects of exchange rate changes on cash and cash equivalent	(197)	(79)	144	94	(80)
<b>Net increase (decrease) in cash and cash equivalents</b> .....	<b>59,424</b>	<b>(27,044)</b>	<b>(4,620)</b>	<b>3,928</b>	<b>(25,541)</b>
Cash and cash equivalents at January 1.....	17,435	76,859	49,815	49,815	45,195
<b>Cash and cash equivalents at December 31 or September 30, respectively</b> .....	<b>76,859</b>	<b>49,815</b>	<b>45,195</b>	<b>53,743</b>	<b>19,654</b>

**a. Cash flows from operating activities**

***Nine months ended September 30, 2018 as compared to nine months ended September 30, 2017***

Cash-outflow from operating activities amounted to CHF 33.0 million for the nine months ended September 30, 2018, an increase of CHF 1.1 million from a cash-outflow of CHF 31.9 million for the nine months ended September 30, 2017, primarily due to increased operating activities in development, marketing and sales, partly offset by higher net sales.

***Financial year ended December 31, 2017 as compared to financial year ended December 31, 2016***

Cash-outflow from operating activities amounted to CHF 39.6 million for the financial year ended December 31, 2017, an increase of CHF 12.5 million from a cash-outflow of CHF 27.1 million for the financial year ended December 31, 2016, primarily due to increased operating activities in development, marketing and sales, partly offset by higher net sales.

***Financial year ended December 31, 2016 as compared to financial year ended December 31, 2015***

Cash-outflow from operating activities amounted to CHF 27.1 million for the financial year ended December 31, 2016, an increase of CHF 4.7 million from a cash-outflow of CHF 22.4 million for the financial year ended December 31, 2015, primarily due to increased operating activities in development, marketing and sales, partly offset by higher net sales.

**b. Cash flows from investing activities**

***Nine months ended September 30, 2018 as compared to nine months ended September 30, 2017***

Cash-inflow from investing activities amounted to a cash inflow of CHF 8.7 million for the nine months ended September 30, 2018, a difference of CHF 30.0 million from a cash-outflow of CHF 21.4 million for the nine months ended September 30, 2017. The difference was primarily due to investment in other short-term financial assets (CHF 12.9 million) and the deposit in escrow of funds (restricted cash) for interest due on our CHF 60 million Senior Unsecured Convertible Bonds 2017-2022 issued in February 2017 (CHF 7.5 million) and, to a lesser extent, to leasehold improvements for our new headquarter in Pratteln. In 2018, we undertook a disposal of other financial assets short-term in the amount of (CHF 7.0 million) and restricted cash (CHF 3.0 million) was partly divested.

***Financial year ended December 31, 2017 as compared to financial year ended December 31, 2016***

Cash-outflow from investing activities amounted to CHF 22.2 million for the financial year ended December 31, 2017, an increase of CHF 21.8 million from a cash-outflow of CHF 0.5 million for the financial year ended December 31, 2016. The increase was primarily due to investment in other short-term financial assets and the deposit in escrow of funds for interest due on our Bonds issued in February 2017, and to a lesser extent to leasehold improvements for our new headquarter in Pratteln.

***Financial year ended December 31, 2016 as compared to financial year ended December 31, 2015***

Cash-outflow from investing activities amounted to CHF 0.5 million for the financial year ended December 31, 2016, a decrease of CHF 0.1 million from a cash-outflow of CHF 0.6 million for the financial year ended December 31, 2016.

**c. Cash flows from financing activities**

***Nine months ended September 30, 2018 as compared to nine months ended September 30, 2017***

Cash-outflow provided by financing activities amounted to CHF 1.2 million for the nine months ended September 30, 2018, a difference of CHF 58.3 million from a cash-inflow of CHF 57.1 million for the nine

months ended September 30, 2017, primarily due to the issuance of our Bonds in February 2017, whose net proceeds amounted to CHF 57.3 million.

***Financial year ended December 31, 2017 as compared to financial year ended December 31, 2016***

Cash-inflow provided by financing activities amounted to CHF 57.1 million for the financial year ended December 31, 2017, an increase of CHF 56.5 million from a cash-inflow of CHF 0.6 million for the financial year ended December 31, 2016, primarily due to the issuance of our Bonds in February 2017, whose net proceeds amounted to CHF 57.3 million.

***Financial year ended December 31, 2016 as compared to financial year ended December 31, 2015***

Cash-inflow provided by financing activities amounted to CHF 0.6 million for the financial year ended December 31, 2016, a decrease of CHF 82.0 million from a cash-inflow of CHF 82.6 million for the financial year ended December 31, 2015, primarily due to the fact that we did not raise equity capital in the financial year ended December 31, 2016, and a decrease in the proceeds from option exercises during such financial year.

**3. Contractual obligations and commitments and contingencies**

The following table sets forth our significant contractual obligations and commitments as of December 31, 2017:

in CHF thousands (unaudited)	Payments due by period (as of December 31, 2017)			
	Less than 1 year	1-5 years	More than 5 years	Total
Convertible bonds <sup>(1)</sup> .....	0	60,000	0	60,000
Operating leases .....	1,176	1,177	34	2,387
<b>Total</b> .....	<b>1,176</b>	<b>61,177</b>	<b>34</b>	<b>62,387</b>

(1) Excluding interest payments.

Pursuant to the Option Agreement with Idorsia entered into on November 20, 2018, we have agreed to pay to Idorsia USD 20.0 million in cash as part of the consideration for the acquisition of the option for the exclusive sub-license relating to ReveraGen’s vamorolone, which we intend to fund from the net proceeds of the issuance and sale of the Offered Shares. In addition, we may be required to make certain payments under license and collaboration agreements. For more information on these agreements and our contingent obligations thereunder see “—*Operating expenses*” beginning on page 45.

As part of our ordinary course of business, we have entered into several contracts for, e.g., clinical or technical development services and the manufacturing of active pharmaceutical ingredients and finished drug products. Commitments are within current market prices and can be terminated at our discretion.

**a. Convertible bonds**

In February 2017, the Company issued an aggregate of CHF 60 million Senior Unsecured Convertible Bonds 2017-2022 (see “—*CHF 60 million Senior Unsecured Convertible Bonds 2017-2022*” in “*The Company and its Business*” beginning on page 88 for more information). The Bonds carry interest at 5% *per annum* and will mature in February 2022, to the extent not converted or redeemed earlier, as provided by the terms and conditions of the Bonds. Out of the net proceeds of the Bonds, an amount corresponding to the interest payable on the Bonds for the first three years of their term was put into escrow to be used for interest payments.

**b. Operating leases**

We are leasing all of our premises as well as some vehicles for staff where necessary. As of December 31, 2017, our aggregate commitments under such leases amounted to CHF 2.4 million. This figure includes the commitments under the lease for our new headquarter in Pratteln, Switzerland.

**c. Off-balance sheet arrangements**

There are no off-balance sheet arrangements.

**F. Quantitative and qualitative disclosures about market risk**

**1. Foreign exchange rate risk**

We hold significant cash amounts in four major currencies, Swiss franc, euro, U.S. dollar, and British pound, to cover the majority of future expected expenses. Evaluations based on market values are performed regularly. Any fair value changes of such currency positions are recorded accordingly in the income statement. Our primary exposure to financial risk is due to fluctuation of exchange rates between the Swiss franc and euro, and to a limited extent, between the Swiss franc and U.S. dollar and British pound, respectively. In order to reduce our foreign exchange rate exposure, we may decide to enter into derivative currency contracts (forwards, options, structured derivatives) in the future to hedge against additional major foreign currency exchange rate fluctuations. Currently, we have no foreign exchange hedge contracts.

As of December 31, 2017, a 5% strengthening (weakening) of the euro against the Swiss franc, with all other variables held constant, would have had a positive (negative) effect on our result before taxes of CHF 0.4 million. As of December 31, 2016, a 5% strengthening (weakening) of the euro against the Swiss franc, with all other variables held constant, would have had a positive (negative) effect on our result before taxes of CHF 0.2 million. There is no impact on the Group's equity.

**2. Interest rate risk**

We hold our cash on deposit/current accounts or invest cash through money market instruments in line with or treasury guidelines in order to satisfy our financial needs over time.

As of December 31, 2017, an increase of the market interest rate by 50 basis points, with all other variables held constant, would have had an effect on our result before taxes of CHF 0.3 million (as per December 31, 2016: CHF 0.2 million; as per December 31, 2015: CHF 0.4 million). A decrease of the market interest rate by 50 basis points would have had the opposite effect of equal magnitude.

**3. Credit default risk**

We have a certain concentration of credit default risk. Short term investments are invested in cash on deposit and in low-risk money market funds (money market accounts with top tier banks or highly rated money market investment instruments) that offer daily liquidity. No investment or contract with any single counterparty, except cash on deposit subject to the criteria above, comprises more than 30% of cash and cash equivalents at the date of investment.

We have policies in place to ensure that sales of products or entered partnerships are made to or entered with customers or partners with an appropriate credit history and a commitment to ethical business practices. The maximum credit risk exposure is limited to the carrying amount of its financial assets including derivatives.

#### **4. Liquidity risk**

Prudent liquidity risk management implies maintaining sufficient cash and cash equivalents. Currently, we are financed through equity and our CHF 60 million Senior Unsecured Convertible Bonds 2017-2022. There is no other interest-bearing funding through debt instruments. We calculate the needs for aligning the current expenses against the need for optimized financial investments on a rolling basis.

#### **5. Capital management**

The first priority of our capital management is to provide adequate cash funds to ensure the financing of successful development and marketing activities so that future profits can be generated by gaining marketing authorization approvals for pharmaceutical products. As a company with currently one product in a small market, the capital management continues to be focused on the cash and cash equivalents position and is governed by specific Group treasury guidelines.

The funds raised through past equity issuances of the Company and through the Bonds, together with funds generated through product sales, enabled the Group to be adequately financed.

No changes in goals and policies of the treasury management have been made during the past two reporting years.

#### **G. Significant accounting policies**

Our reported financial condition and results of operations are sensitive to accounting principles, methods and assumptions that form the basis for the preparation of our consolidated and statutory financial statements. Our accounting policies, the judgments we make in the establishment and application of these policies, and the sensitivity of reported results to changes in accounting policies and assumptions are factors to be considered along with our consolidated and statutory financial statements. For a discussion of our significant accounting policies, see Note 2 to our 2016 Consolidated Financial Statements and Note 2 to our 2017 Unaudited Condensed Consolidated Financial Statements included elsewhere in this Offering Memorandum.

In accordance with the Swiss Federal Act on Occupational Old Age, Survivors' and Invalidity Pension Provision of June 25, 1982 (as amended), our Swiss employees are affiliated with a collective independent pension fund. In accordance with IFRS, we apply IAS 19 with respect to our long-term obligations under Swiss pension plans. The pension plans under which we provide retirement benefits to our Swiss employees are operated by a foundation and funded by defined monthly contributions from both the employer and employee. Under Swiss pension plans, employers' obligations are limited to paying the monthly contribution except in the event of a structural underfunding. As an employer would be required to contribute to a restructuring in the event of a structural underfunding, Swiss pension plans are generally classified as defined benefit plans under IFRS. In addition, IFRS generally applies more conservative actuarial assumptions with respect to the funded status of pension plans than would be the case under local Swiss GAAP, resulting in a lower percentage of a pension plans' funded status and, in turn, the recording of a higher expense for the provisions relating to long-term pension obligations than would be the case if the "risk sharing" concept between employer and employee were recognized for such Swiss pension plans under IAS 19. As of December 31, 2017, the present value of obligations under our Swiss pension plans calculated in accordance with IFRS was CHF 24.2 million and the fair value of assets was CHF 15.8 million, resulting in a net defined obligation of CHF 8.4 million.

#### **H. Critical accounting judgments, estimates and assumptions**

The preparation of our consolidated and statutory financial statements requires management to make certain estimates and apply judgements that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of sales and

expenses during the reporting period. We base our estimates and judgements on historical experience, current trends and other factors that management believes to be important at the time the financial statements are prepared. Due to the need to make estimates about the effect of matters that are inherently uncertain, materially different amounts could be reported under different conditions or using different assumptions.

Management has changed, and might from time to time change, the Group's accounting policies, including in order to comply with new requirements or to potentially adopt policies more in line with other industry participants, so as to allow better comparability. Any change in accounting policies could impact reported results for prior and future periods. Changes in current economic conditions and other events may also have a material impact on the actual figures. Therefore, actual results could differ from our estimates. The following areas involve assumptions and estimates that can have a significant impact on the consolidated financial statements:

**1. Measurement and impairment testing of intangible assets and inventory**

Our main intangible assets relate to Raxone<sup>®</sup>. We perform annual impairment testing with regard to this asset using the risk-adjusted net present value model ("rNPV"), taking into consideration the expected cumulative probability of reaching the market to calculate recoverable amount. For as long as we have marketing authorization for Raxone<sup>®</sup>, we do not conduct a specific impairment testing on our inventory (which predominantly comprises raw material (active pharmaceutical ingredients) and semi-finished goods relating to Raxone<sup>®</sup>). We had to impair the intangible assets relating to Raxone<sup>®</sup> in 2012. After receipt of marketing authorization for Raxone<sup>®</sup> in LHON for the EU, in 2015 we reversed the previous impairment in the amount of CHF 26.2 million and transferred the related intangible assets into assets available for use with a useful life of 10 years. Amortization of such intangible assets began in September 2015. Also in 2015, we reversed the impairment on some inventories in the amount of CHF 0.9 million.

**2. Personnel expenses from share-based payments**

We value personnel expenses resulting from share-based payments in accordance with IFRS 2. We determine the fair value of granted stock options and stock-appreciation rights at each grant date by using the Hull-White pricing model, using the parameters set forth in note 17 to the 2017 Consolidated Financial Statements included elsewhere in this Offering Memorandum. We recognize the so-determined value as personnel expense over the period during which we receive services from the respective individual.

**3. Actuarial valuations in the context of defined benefit pension plans**

We calculate assets and liabilities under defined benefit pension plans in accordance with IAS 19 on the basis of assumptions, such as on discount rates, salary increases and pension mortality rates. We assess and adjust these assumptions on an annual basis. Actuarial valuations under such pension plans are associated with significant uncertainties due to the long-term nature of the plans.

**I. New, revised or amended IFRS standards**

See Note 2 to the 2017 Unaudited Consolidated Financial Statements included elsewhere in this Offering Memorandum for a list of new, revised or amended IFRS standards that have become effective and did not have any significant impact on our consolidated financial statements or that have been published but are not yet effective and have not been adopted early by us. Currently, based on our preliminary analysis, we do not expect any significant impact on the consolidated financial statements from these new, revised or amended standards, with the exception of IFRS 16.

IFRS 16 – Leases introduces a single lessee accounting model requiring lessees to recognize right-of-use assets and lease liabilities for leases with a term of more than twelve months and thus eliminates the current classification model for lessee's lease contracts as either operating or finance leases. This will bring the previous off-balance sheet leases on the balance sheet in a manner largely comparable to current finance lease accounting. Adoption of IFRS 16 will result in us recognizing right-of-use assets and lease liabilities

for all contracts with a term of more than twelve months that are, or contain, a lease. For leases currently classified as operating leases, we currently do not recognize related assets or liabilities, and instead spread the lease payments over the lease term on a linear basis and disclose the total commitment in the notes to our annual consolidated financial statements. We currently expect to capitalize our leasing arrangements relating to real estate and cars under IFRS 16. We will continue to assess the potential effect of IFRS 16 on our consolidated financial statements in 2018.

## **J. Recent developments**

On November 20, 2018, we announced the signing of an agreement (the “**Option Agreement**”) with Idorsia Pharmaceuticals Ltd, Allschwil, Switzerland, under which we have acquired an option to obtain from Idorsia an exclusive sub-license to commercialize the active ingredient vamorolone, a non-hormonal steroid modulator for the treatment of DMD developed by ReveraGen BioPharma, Inc., a U.S.-based company. See “X. *The Company and Its Business—E. Additional information on our business—1. Material agreements—a. License and collaboration agreements—Option Agreement with Idorsia*” beginning on page 84.

On December 11, 2018, the shareholders of the Company resolved on an ordinary capital increase of up to CHF 5,000,000 by issuing a maximum of 5,000,000 new shares in the Company with a par value of CHF 1 each (the “**Offered Shares**”). The Company intends to use the net proceeds of this Offering to fund the USD 20.0 million payable to Idorsia Pharmaceuticals Ltd as the cash component of the consideration for the acquisition of the option for the exclusive sub-license to commercialize ReveraGen’s vamorolone for the treatment of DMD as well (see “—*License and collaboration agreements*” in “*The Company and its Business*” beginning on page 84 for more information) and any net proceeds from the Offering in excess of that amount for general corporate purposes. Upon completion of the Offering, assuming that all 5,000,000 Offered Shares are sold in the Offering, the share capital of the Company will be CHF 12,527,479 and consist of 12,527,479 Shares.

As part of its business expansion strategy, Santhera is actively exploring the possibility of entering into commercial agreements with third-party companies for the commercialization (marketing, distribution and sales) of drugs approved for the treatment of diseases within Santhera’s therapeutic focus areas (*i.e.*, neuromuscular, neuro-ophthalmology and pulmonary diseases) in Europe. In doing so, Santhera would leverage its existing commercial infrastructure in Europe and expand its commercial-stage product pipeline to generate additional income from product sales with only limited additional investments in adapting its existing commercial infrastructure.

In this context, Santhera is currently in advanced discussions with a pharmaceutical company to explore the possibility of supporting this company in the commercialization of a rare-disease medication for the treatment of a neuromuscular disease for which a marketing authorization is expected to be granted near-term. Commercial support would take the form of handling marketing and sales for the company’s product in a number of European countries in exchange for a fraction of the net sales. Santhera does not expect that the agreement, if reached, will require it to make any material investment into the adaptation of its commercial operations, nor does it expect material income from sales near term, due to the time needed to achieve pricing and reimbursement on a country-by-country basis. However, we believe that overall such commercial agreement for a novel treatment to address an unmet medical need for a rare neuromuscular disease would increase our visibility as a commercial-stage company, would add a second marketed product to our pipeline and would allow our commercial teams to engage with key opinion leaders and treating physicians in preparation of the future launch of idebenone and vamorolone for the treatment of DMD. There can be no assurance that the current discussions will be successful and that an agreement will be reached in the near term or at all.

## X. THE COMPANY AND ITS BUSINESS

### A. Business overview

We are a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. The portfolio comprises clinical stage treatments and a marketed treatment for neuro-ophthalmologic, neuromuscular and pulmonary diseases. We believe that we have developed significant expertise in the understanding of the underlying causes of these diseases and our goal is to become a market leader in the development and commercialization of products for their treatment.

Our lead compound is idebenone, an anti-oxidant agent that we believe can help improve production of energy by mitochondria in certain patients whose mitochondrial function is impaired by genetic defects. Idebenone is the active ingredient in Raxone<sup>®</sup>, our product marketed in the EU and Israel for the treatment of Leber's hereditary optic neuropathy ("LHON"), and a potential active ingredient or product candidate for the treatment of various mitochondrial, neuromuscular and neuro-ophthalmological diseases. Idebenone is a drug that has been well-known for decades, with a well-known mode of action and a well-established safety profile. Idebenone's mode of action suggests the potential of a wide therapeutic application.

In November 2018, we entered into an agreement (the "**Option Agreement**") with Idorsia Pharmaceuticals Ltd ("**Idorsia**") under which we have acquired an option for the exclusive sub-license to commercialize ReveraGen BioPharma, Inc.'s ("**ReveraGen**") vamorolone, a non-hormonal steroid modulator developed by ReveraGen and licensed to Idorsia. We will be able to exercise this option against payment of USD 30.0 million at the latest when the data from the Phase IIb study in Duchenne muscular dystrophy ("**DMD**") patients are available, which is expected to be the case in 2020 according to the current development plan. As initial consideration for the acquisition of the option for the exclusive sub-license relating to ReveraGen's vamorolone, we issued the 1,000,000 Idorsia Shares to Idorsia and have agreed to pay USD 20.0 million in cash. Both the issuance of the 1,000,000 Idorsia Shares to Idorsia and the payment of the USD 20.0 million in cash are non-refundable. The cash amount is intended to compensate Idorsia for having already paid USD 15.0 million to ReveraGen to fund the ongoing Phase IIb trial in patients with DMD.

Currently, we are focusing our development and commercialization efforts on the following product and product candidates:

- *Our marketed product: Raxone<sup>®</sup> in LHON.* LHON is a rare, severe hereditary eye disease affecting primarily men in their 20s and 30s and leading to central vision loss in both eyes. Raxone<sup>®</sup> is, to our knowledge, the first product approved for the treatment of LHON. We received marketing authorization for Raxone<sup>®</sup> in LHON in all 28 EU countries, Norway, Iceland and Liechtenstein in September 2015, and our exclusive distributor received marketing authorization for it in Israel in August 2017. In addition, we filed a marketing authorization application ("**MAA**") for Raxone<sup>®</sup> in LHON in South Korea, which was accepted for review in June 2018.
- *Our lead product candidate: Raxone<sup>®</sup> in Duchenne muscular dystrophy ("**DMD**").* DMD is one of the most common types of inherited degenerative muscle weakness. With symptoms starting at young age, patients commonly are unable to walk by their teenage years and require mechanical ventilation to prolong survival beyond their late teenage years. In the EU, we filed an MAA with the EMA for Raxone<sup>®</sup> in certain patients with DMD with declining respiratory function who are not receiving steroids. However, the EMA's Committee for Medicinal Products for Human Use ("**CHMP**") issued a negative opinion on our MAA, i.e., it recommended that the European Commission not grant us conditional marketing authorization for the treatment of DMD, in September 2017 and maintained such negative opinion in January 2018 after a re-examination procedure. Such negative opinion does not impact the marketing authorization of Raxone<sup>®</sup> for the treatment of LHON. In July 2018, we announced results of a comparative analysis of the Phase III DELOS clinical trial outcome and new data from natural history studies. This analysis showed that the treatment effect with idebenone observed in the Phase III DELOS clinical trial can be linked to a delay in the

initiation of assisted ventilation by three years, which is of high clinical relevance. We and our academic partners intend to prepare for the publication of additional clinical data relating to the long-term efficacy of idebenone on respiratory function outcomes in patients with DMD in the coming months, supporting the positive data from the successful Phase III DELOS clinical trial. We plan to discuss the findings with regulators in the coming months and to include them in the regulatory dossier in preparation of MAAs for idebenone in DMD in Europe and the U.S. in 2019.

- In the U.S., we have received fast track designation for Raxone® in DMD from the U.S. Food & Drug Administration (“FDA”). In addition, we are currently conducting a phase III clinical trial with certain DMD patients with declining respiratory function who are receiving steroids and we currently expect top line data from this trial in 2020.
- *Vamorolone in DMD.* We intend to develop vamorolone for early stage DMD patients requiring an anti-inflammatory, muscle strengthening glucocorticoid before onset of respiratory decline. We expect the combination of vamorolone and idebenone to address the medical needs of DMD patients at all disease stages. ReveraGen has conducted extensive non-clinical studies, Phase Ia and Ib studies and Phase IIa and IIa-extension studies of vamorolone. Based on knowledge obtained from these studies, ReveraGen is currently conducting the Phase IIb - VISION DMD trial, which together with the previous studies could form the basis for approval of vamorolone in DMD.
- *Our early stage product candidate, omigapil in congenital muscular dystrophy (“CMD”).* CMD is a group of inherited conditions that causes progressive and potentially life-threatening loss of muscle tissue, affecting frequently newborns and children. We are exploring the compound omigapil for the treatment of CMD in children and adolescents. In April 2018, we announced that the top line data of a phase I clinical trial for omigapil in CMD (CALLISTO) that we conducted in collaboration with the National Institute of Neurological Disease and Strokes (the “NINDS”), an institute within the National Institutes of Health (the “NIH”) in the U.S., suggest that the trial met its primary objective to establish a favorable pharmacokinetic profile of omigapil and demonstrated that the drug was safe and well tolerated in the children and adolescents that participated in the trial. Further development is currently being discussed with clinical experts and regulators.
- *Our early stage product candidate, POL6014 in cystic fibrosis (“CF”).* CF is a rare, life-threatening, progressive genetic disease that is typically diagnosed in young children and affects primarily the lungs but also the digestive system. In February 2018, we in-licensed the compound POL6014 that we believe has the potential to treat CF and other neutrophilic lung diseases. Based on prior development work by Polyphor, including two phase I clinical trials, we have started a phase I multiple ascending dose (“MAD”) clinical trial of POL6014 in CF in the fourth quarter of 2018.

We have entered into a number of strategic development collaborations, by in-licensing and co-developing promising product candidates, and established commercial relationships with distributors to exploit the commercial potential of our products. We believe that these collaborations demonstrate our ability to successfully partner with global pharmaceutical and biotechnology companies and to establish ourselves as an attractive development and commercialization ally.

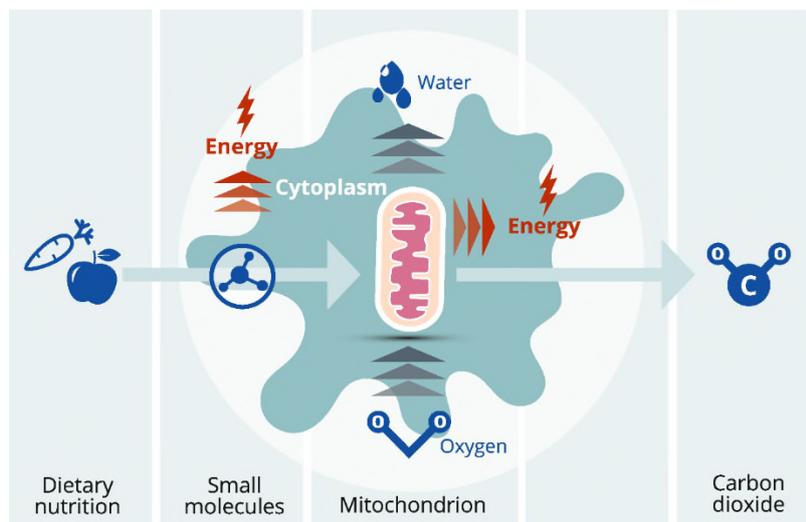
We are managed by a capable, experienced and professional team, with the members of our Executive Management alone having more than 80 years of combined pharmaceutical industry experience. See “Board of Directors and Executive Management” beginning on page 122.

Santhera in its present form was founded in September 2004. The Shares have been listed on the SIX Swiss Exchange since November 2006. Our headquarters are located in Pratteln, Switzerland, with subsidiaries in Switzerland, Germany, the United Kingdom, Italy, the Netherlands, Spain, Liechtenstein, the U.S., Canada and Finland. As of September 30, 2018, we had 119 employees (113.6 full-time equivalent). These figures are expected to increase in the near future due to our ongoing expansion of operations relating to the commercialization of Raxone®.

## B. About mitochondrial diseases and our lead compound idebenone

Mitochondria are found within virtually every cell of the body and generate energy required by the cells to function. They are sometimes colloquially referred to as the powerhouse of the cell.

Mitochondrial diseases are often a result of inherited genetic mutations and typically affect organs with high energy requirements, such as the brain, muscles, eye, ear, heart, liver and the gastrointestinal tract. When mitochondria do not produce enough energy, cells within the organ do not function properly, become damaged and eventually die, resulting in the symptoms typically seen in mitochondrial diseases.



**Figure 1: Functioning of mitochondria**

Our marketed product is Raxone<sup>®</sup>, an oral formulation of the compound idebenone, which is chemically a synthetic short-chain benzoquinone and cofactor for an enzyme called NAD(P)H:quinone oxidoreductase (NQO1). Idebenone has a dual mode of action: studies conducted by us as well as by third parties have shown that idebenone improves the flow of electrons within the mitochondrial electron transport chain, thereby enhancing mitochondrial function, and that it also acts as a cell-protecting antioxidant. Numerous diseases exist with respect to which a defect in the mitochondrial electron transport chain and the resulting increase in oxidative stress (the presence of an excessive amount of so-called reactive oxygen species, commonly known as free radicals, which are by-products of the cell metabolism that may damage cells) caused by such defect is considered to be an underlying cause of such diseases.

We believe that idebenone's pharmacological properties make it a development candidate of choice to treat diseases with underlying mitochondrial dysfunction. For example, based on the results of our clinical trials of Raxone<sup>®</sup> in LHON, we believe that Raxone<sup>®</sup> may reactivate nerve cells in the retina (retinal ganglion cells) that are still viable but no longer active by helping bypass the deficient mechanism that prevents the mitochondria from working properly. In addition, based on the results of our phase III clinical trial with certain DMD patients who were not also being treated with steroids, we believe that Raxone<sup>®</sup> may slow the loss of respiratory function to which the malfunction of the mitochondria that is associated with DMD may contribute.

Moreover, we demonstrated in multiple clinical trials that idebenone has a favorable safety profile; the most commonly reported adverse reactions to idebenone are mild to moderate diarrhea (usually not requiring the discontinuation of the treatment), nasopharyngitis (common cold), cough and back pain.

## C. Pipeline and strategy

We are focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. Our portfolio comprises clinical stage treatments and a marketed

treatment for neuro-ophthalmologic, neuromuscular and pulmonary diseases. Our goal is to become a market leader in the development and commercialization of products for treatment of such diseases. We are exploring Raxone®'s clinical and commercial potential in LHON and DMD. In addition, we are investigating omigapil in CMD. The following graph summarizes the status of our pipeline:

Santhera Pipeline <sup>(1)</sup>	Drug	Pre-Clinical	Phase I	Phase II	Phase III	MAA <sup>(2)</sup> Filing	Market
<b>Neuro-ophthalmological Diseases</b>							
 Leber's Hereditary Optic Neuropathy	Idebenone						Raxone® <sup>(3)</sup>
<b>Neuromuscular Diseases</b>							
Duchenne Muscular Dystrophy (GC <sup>(4)</sup> non- users)	Idebenone						
Duchenne Muscular Dystrophy (GC <sup>(4)</sup> users)	Idebenone				(5)		
 Congenital Muscular Dystrophy	Omigapil		(6)				
<b>Pulmonary Diseases</b>							
Cystic Fibrosis	POL6014		(7)				
Alpha-1 Antitrypsin Deficiency	POL6014		To be explored (8)				
Non-Cystic Fibrosis Bronchiectasis	POL6014						
Primary Ciliary Dyskinesia	POL6014						

**Figure 2: Our current pipeline**

- (1) This graph sets out our current pipeline in summary form. For further detail, please refer to the information set out elsewhere in this Offering Memorandum, including in this section “*The Company and its Business*”.
- (2) Marketing Authorization Application.
- (3) Raxone® (150 mg idebenone) is currently approved for the treatment of visual impairment in adolescent and adult patients with LHON in the EU. Under our marketing authorization in the EU, we are required to conduct several post-authorization measures, which include conducting an additional phase IV clinical trial on the long-term effects and safety of Raxone® in LHON, a comparative natural history study, and maintaining a registry of LHON patients treated with Raxone®.
- (4) Glucocorticoids.
- (5) We are currently enrolling patients for a phase III clinical trial for Raxone® in certain DMD patients with declining respiratory function who are receiving steroids (SIDEROS), whose top line data we currently expect to be available in 2020.
- (6) We announced top line data of the phase I clinical trial for omigapil in CMD in April 2018. Further development is currently being discussed with clinical experts and regulators.
- (7) We recently started a phase I multiple ascending dose (MAD) clinical trial of POL6014 in cystic fibrosis.
- (8) We are currently evaluating further development of POL6014 in these indications.

Our strategy includes the following key elements:

***Complete development and obtain marketing authorization for Raxone® in DMD in the EU, the U.S., and Switzerland***

In the EU, we filed an MMA with the EMA for Raxone® in certain patients with DMD with declining respiratory function who are not receiving steroids. However, the CHMP issued a negative opinion on our MAA, i.e., it recommended that the European Commission not grant us conditional marketing authorization for the treatment of DMD, in September 2017 and maintained such negative opinion in January 2018 after a re-examination procedure. Such negative opinion does not impact the marketing authorization of Raxone® for the treatment of LHON.

In July 2018, Santhera announced results of a comparative analysis of the Phase III DELOS trial outcome with new data from natural history studies. This analysis showed that the treatment effect with idebenone

observed in the DELOS trial can be linked to a delay in the initiation of assisted ventilation by three years, which is of high clinical relevance. In the coming months, Santhera and its academic partners will prepare for the publication of additional clinical data relating to the long-term efficacy of idebenone on respiratory function outcomes in patients with DMD, supporting the positive data from the successful Phase III DELOS trial. The findings will be discussed with regulators in the coming months and will be included in the regulatory dossier in preparation of MAAs for idebenone in DMD in Europe and the U.S. in 2019.

Further, we plan to complete our ongoing phase III clinical trial for patients with DMD who are receiving steroids (SIDEROS), and we currently expect top line data from this trial to be available in 2020. Should the primary endpoint of the SIDEROS trial be met, we intend to seek to expand a potential marketing authorization in the EU and/or Switzerland for Raxone<sup>®</sup> in DMD to cover patients who are receiving steroids. We previously received fast track designation for Raxone<sup>®</sup> in DMD from the FDA, which means that we may benefit from an expedited development and/or review process. As a result, we intend to request priority review when we file a new drug application (“NDA”) for Raxone<sup>®</sup> for the treatment of DMD patients who are not receiving steroids with the FDA. We expect to base our NDA on the data from our phase III clinical trial of Raxone<sup>®</sup> in certain DMD patients with declining respiratory function who were not being treated with steroids (DELOS). However, there is considerable uncertainty around whether the FDA would accept an NDA filing based on the limited data from such clinical trial.

***Participate in the commercialization of Raxone<sup>®</sup> in DMD and other product candidates, if and when marketing authorization is granted***

We intend to keep building the commercial infrastructure in Europe, the U.S. and other geographic markets necessary to support the commercialization of our product candidates if and when we believe a marketing authorization in a particular geographic market appears imminent. We are operating in the rare disease area in which patients typically form local, national and global patient groups that are well informed concerning the latest treatment possibilities. As a result, physicians can be located and targeted efficiently by a small and highly skilled in-house team or by our distribution partners.

Where appropriate, we may in the future elect to work with strategic partners, distributors and/or contract sales forces in order to assist us in the commercialization of our products. In certain instances, we may consider building our own commercialization infrastructure.

***Maximize the value of Raxone<sup>®</sup> in LHON in the EU and elsewhere***

We plan to continue to seek reimbursement by third-party payers for Raxone<sup>®</sup> in LHON in various EU countries.

As of the date of this Offering Memorandum, full reimbursement of Raxone<sup>®</sup> in LHON has been achieved for Germany, the Netherlands, Italy, Sweden, Scotland and six other jurisdictions. In several other jurisdictions, including France and England and Wales, Raxone<sup>®</sup> in LHON is currently covered by special reimbursement schemes. As an example, the Scottish Medicines Consortium (“SMC”) accepted Raxone<sup>®</sup> for restricted use by NHS Scotland in the treatment of visual impairment in adolescent and adult LHON patients. We believe this to be a significant decision, as the SMC is a well-known reference to other pricing and reimbursement bodies in Europe.

Also, we plan to expand our marketing and sales operations in those EU countries where we distribute Raxone<sup>®</sup> ourselves (see “—*Presence in Europe*” beginning on page 89 for more information on our distribution arrangements in Europe). Further, we plan to continue to commercialize Raxone<sup>®</sup> for the treatment of LHON in Israel via the third-party distributor Megapharm Ltd. (in August 2017, Megapharm Ltd. obtained marketing authorization in Israel).

If and to the extent we obtain marketing authorization for Raxone<sup>®</sup> in LHON in other jurisdictions, we will commercialize Raxone<sup>®</sup>, including seeking reimbursement and arranging for its distribution in those jurisdictions.

***Advance the development of our clinical product candidates, POL6014 for CF and omigapil for CMD***

Based on prior development work by Polyphor, including phase I trials in healthy volunteers and CF patients, we have started a multiple ascending dose (MAD) phase Ib trial in patients with CF in the fourth quarter of 2018. Likewise, we are currently in discussions with clinical experts and regulators relating to further development of omigapil in CMD.

***Leverage our experience and relationships to selectively in-license additional promising product candidates***

We intend to expand our portfolio of product candidates by selectively in-licensing or acquiring other promising product candidates or compounds for the treatment of diseases that have a severe impact on the lives of the people affected by them, currently lack treatment options and are likely to qualify for orphan drug status, especially in the neuro-ophthalmologic, neuromuscular and pulmonary areas. In particular, we are continuously looking at opportunities to leverage our expertise in the development of product candidates targeting such diseases and in achieving market exclusivity and fast track, break-through medicine and similar designations for other compounds and/or indications for which we may in-license or acquire development and/or commercialization rights, as well as to achieve synergies in the distribution of our marketed product and any future products for which we may receive marketing authorization. In this context, we are currently evaluating several potential in-licensing and acquisition opportunities in our three primary therapeutic areas.

**D. Our product and product development portfolio**

**1. Our marketed product: Raxone® in LHON**

**a. Leber's hereditary optic neuropathy (LHON)**

LHON is a rare, severe hereditary eye disease that usually affects young, otherwise healthy individuals and is more common in men than women. On average, LHON onsets at an average age between 27 and 34 years. Loss of central vision occurs in the majority of patients within one year of their symptoms becoming apparent. The loss of vision in the first eye is typically sudden, abrupt, painless and profound. This is typically followed by loss of vision in the other eye 1–3 months later. The rapid loss of vision caused by LHON, which in most cases leads to profound loss or severe deterioration of vision, has a dramatic impact on the quality of life for patients, and is associated with considerable economic and social costs due to productivity loss, lower employment rates, income loss and costs of care.

LHON is caused by mutations in genes in the mitochondria. When such mutations are present, nerve cells in the retina at the back of the eye (so-called retinal ganglion cells) do not have enough energy to work properly, leading to loss of vision.

**b. Market opportunity**

The prevalence (number of cases during a particular period or at a particular point in time) of LHON has been estimated to be two per 100,000 individuals in Europe. However, only a fraction of these patients still have viable retinal ganglion cells and could therefore benefit from treatment with Raxone®. Since LHON is an extremely rare disease, we believe that the market for Raxone® in LHON is small (see risk factor “*Our marketed product, Raxone® in LHON, will not allow us to become profitable. Our future profitability, if any, will depend on us being able to obtain marketing authorization and, thereafter, pricing and reimbursement approvals for our product candidates, in particular Raxone® in DMD, as well as potentially in other indications.*” beginning on page 13).

**c. Treatment of LHON with Raxone®**

Our product Raxone® is to our knowledge the first and only approved treatment for LHON, and has been approved in the EU and Israel for the treatment of visual impairment in adolescents and adults with LHON. Based on the results of our clinical trials we believe that Raxone® helps bypass the deficient mechanism caused by the mutated genes that prevents the mitochondria from working properly. As a result, Raxone® may reactivate nerve cells in the retina (retinal ganglion cells) that are still viable but no longer active. In addition, studies conducted by us as well as by third parties have shown that Raxone® also works as a cell-protecting antioxidant, preventing damage induced by so-called reactive oxygen species, commonly known as free radicals, which are by-products of the cell metabolism that may damage cells.

The results of our RHODOS trial (see “—*Clinical Development*” beginning on page 72 for more information) suggest that Raxone® can prevent further vision loss associated with LHON and promote clinically relevant recovery of visual acuity in LHON patients with disease duration of up to five years. An early diagnosis of LHON offers patients the best chance for the treatment of their condition with Raxone® in the long term. There is a window of opportunity for the optimal treatment of LHON with Raxone® when the retinal ganglion cells are still viable; clinical data of our ongoing Expanded Access Program (the “EAP”) (see “—*Clinical Development*” beginning on page 72 for more information) suggest that up to 50% of patients with disease duration of less than one year experience a clinically relevant recovery of visual acuity following Raxone® treatment.

Raxone® is available in the form of film-coated tablets containing 150 mg idebenone.



**Figure 3: Commercial packaging of Raxone®**

In the December 2017 issue of the *Journal of Neuro-Ophthalmology*, a group of experts (a majority of whom disclosed having or having had a business relationship with us) published what is to our knowledge the first International Consensus Statement on the Clinical and Therapeutic Management of LHON. We had no role in the selection of experts or in the preparation, review, or approval of the final list of consensus statements. We partly funded the logistics and organization of the meeting at which the Consensus Statement was elaborated and partly funded participants’ travel expenses, but did not remunerate the participants. The Consensus Statement is intended to provide guidance for the clinical and therapeutic management of LHON. According to the Consensus Statement, there was a strong consensus that treatment with Raxone® should be started as soon as possible in LHON patients with disease duration of less than one year and that such treatment should be continued for at least one year and for up to one year after visual acuity no longer improves. There was also a strong consensus that there is not enough evidence to recommend treatment in LHON patients with disease duration of one to five years, and no evidence to recommend treatment in LHON patients with disease duration above five years. This notwithstanding, we believe that the results of our RHODOS trial suggest that Raxone® can also benefit LHON patients with disease duration of one to five years, and we are aware of a retrospective study conducted by third parties on a small sample of patients

whose results suggest that LHON patients with disease duration above five years may also benefit from treatment with Raxone®.

#### d. Clinical Development

We have demonstrated the efficacy and safety profile of Raxone® in LHON in what is, to our knowledge, the largest clinical development program ever undertaken with regard to a drug for the treatment of LHON. Our clinical development program included a double-blind, randomized, placebo-controlled trial (“**RHODOS**”), an observational long-term follow-up study (“**RHODOS-OFU**”), an EAP, and a natural history case record survey (the “**CRS**”). We conducted a number of additional clinical studies in multiple indications that also support the safety profile of Raxone®.

- RHODOS was, to our knowledge, the first and only randomized, placebo-controlled clinical trial to be completed with regard to a drug for the treatment of LHON. RHODOS was conducted in 85 patients aged 14 to 66 years having all three major genetic mutations causing LHON and with an onset of vision loss within the last five years prior to enrollment.
- RHODOS-OFU was conducted as a single-visit observational follow-up study in 58 patients who had completed the RHODOS trial and who were assessed after a median of 30 months without treatment.
- The EAP aimed at providing insight into Raxone®’s therapeutic potential in a real-world setting and was conducted under the Named Patient Program in the EU and the Investigational New Drugs regulations in the U.S. Enrollment was limited to patients presenting for treatment within one year of onset of symptoms of LHON. Data from 69 patients at 36 centers worldwide were analyzed and reported. The program is still ongoing.
- The CRS is a collaboration between us and the European Vision Institute Clinical Research Network (EVICR.net) in which historically documented visual acuity data from LHON patients not treated with idebenone were collected from participating centers.

#### e. Efficacy

In our RHODOS trial, patients treated with Raxone® on average experienced an improvement of visual acuity with their eye experiencing the most improvement (primary endpoint) by three letters on an eye chart, though this result was not significant over placebo. The difference between the visual acuity in the eye with better visual acuity after treatment with Raxone® compared to the visual acuity in the eye with better visual acuity before such treatment (main secondary endpoint) was 6 letters on an eye chart. Additionally, we conducted an analysis of a subgroup of eight LHON patients with pre-specified degrees of visual acuity, six of whom had been treated with Raxone® in the RHODOS trial, and two had received a placebo. This analysis showed that visual acuity of all six patients who had received Raxone® did not deteriorate in the pre-specified way, whereas visual acuity of both patients who had received a placebo deteriorated. The RHODOS-OFU indicated that the effect of Raxone® may be maintained.

In RHODOS, we also performed an analysis in which we evaluated the proportion of patients who had a clinically relevant recovery of visual acuity (defined as an improvement from being unable to read a single letter on an eye a chart to being able to read at least five letters, or an improvement by at least ten letters) in at least one eye. 30.2% of patients experienced clinically relevant recovery of visual acuity compared with 10.3% in the placebo group after six months of treatment.

According to data from the EAP and the CRS, 30.6% of (Raxone®-treated) patients in the EAP experienced a clinically relevant recovery after six months, compared to 19.1% of patients in the CRS (who had received no treatment for LHON). In the EAP, the number of patients with a clinically relevant recovery and the degree of clinically relevant recovery increased with a longer duration of treatment with Raxone®.

**f. Market exclusivity, regulatory status and sales**

We have been granted orphan drug designation for Raxone<sup>®</sup> for the treatment of LHON in the EU (until September 2025), the U.S., and South Korea. The product is not patent protected.

We received marketing authorization for Raxone<sup>®</sup> for the treatment of adolescents and adults with LHON in all 28 EU countries, Norway, Iceland and Liechtenstein, in September 2015, and our exclusive distributor has received marketing authorization for Raxone<sup>®</sup> in LHON in Israel in August 2017. In addition, we filed an MAA for Raxone<sup>®</sup> in LHON in South Korea and Serbia, which the Korean Ministry of Food and Drug Safety accepted for review in June 2018, and we currently expect a decision from the South Korean drug regulatory authorities in the second quarter of 2019.

Raxone<sup>®</sup> is, to our knowledge, the first therapy approved for treatment of LHON. The marketing authorization for the EU was granted under “exceptional circumstances”. Such authorization may be given when comprehensive efficacy and safety data cannot be obtained, but it is still appropriate to grant the authorization. In the case of Raxone<sup>®</sup> in LHON, authorization under “exceptional circumstances” was given because the CHMP was of the view that it was not feasible to generate a comprehensive data set, mainly due to the rarity of LHON. Furthermore, at the time of the CHMP’s determination, idebenone was already used by physicians on an off-label basis to treat LHON patients, which is why it was believed that neither physicians nor patients would be prepared to participate in a placebo-controlled trial. Under the EU marketing authorization granted to us for Raxone<sup>®</sup> in LHON, we are required to conduct several post-authorization measures. In particular, we are currently conducting an additional phase IV clinical trial on the long-term effects and safety of Raxone<sup>®</sup>, called LEROS, which is an open label interventional study, as well as a second comparative natural history study that builds upon the data obtained from our CRS. In addition, we are maintaining a registry of LHON patients treated with Raxone<sup>®</sup> (called PAROS), as required by the EU marketing authorization.

We launched Raxone<sup>®</sup> for the treatment of LHON in Germany in October 2015. For more information on our distribution arrangements see “—*Distribution and marketing*” beginning on page 89.

Together with our distributors, we currently sell Raxone<sup>®</sup> in LHON in more than 20 countries. In 2016, the first full year of commercialization of Raxone<sup>®</sup> in LHON, we generated net sales of Raxone<sup>®</sup> in LHON in the amount of CHF 19.0 million. In 2017, we generated net sales of Raxone<sup>®</sup> in LHON in the amount of CHF 22.9 million. We generate the majority of our net sales of Raxone<sup>®</sup> in LHON in France and Germany.

**g. Competing therapeutic approaches**

To our knowledge, probably the most advanced product development program for treatment of LHON is based on gene therapy with an ongoing phase III clinical trial being conducted by Gensight Biologics, Paris, France, addressing patients within one year of symptom onset and carrying one of the major genetic mutations causing LHON. A successful proof of concept trial with gene therapy was reported by an academic institution in China on a small number of patients. In addition, Stealth BioTherapeutics, Inc., Newton, Massachusetts, USA, is recruiting patients for a phase II clinical trial in LHON using a mitochondria targeting peptide. We believe that all of these programs are still several years from filing an MAA.

We are aware of a product development program for treatment of LHON based on gene therapy conducted by Gensight Biologics, Paris, France, that addresses patients within one year of symptom onset and carrying one of the major genetic mutations causing LHON. To our knowledge, the completed phase III clinical trial of that program did not meet its primary endpoint (predefined as a +15 ETDRS (Early Treatment Diabetic Retinopathy Study) letters difference in visual acuity between eyes treated with Gensight’s compound and those not treated), but met some secondary endpoints.

## 2. **Our lead product candidate: Raxone<sup>®</sup> in DMD**

### a. **Duchenne muscular dystrophy (DMD)**

DMD is one of the most common and devastating types of muscular degeneration. It is an inherited condition and primarily affects boys starting at an age between three and five years on average. DMD is characterized by a loss of a protein called dystrophin, which links the muscle cytoskeleton and extracellular matrix to maintain muscle integrity. This loss leads to cell damage, an uncontrolled influx of calcium, and a dysfunction of mitochondria associated with reduced energy production in muscle cells. This results in progressive muscle weakness, loss of muscle tissue and early illness and death due to cardio-respiratory failure. Patients are commonly unable to walk by their teenage years. Progressive respiratory muscle weakness leads to restrictive respiratory disease, hypoventilation, ineffective cough, recurrent pulmonary infections, respiratory failure and ultimately the need for daytime ventilation. As respiratory insufficiency develops, mechanical ventilation becomes necessary to prolong the life of the patient beyond the late teenage years.

### b. **Market opportunity**

Based on third-party studies, we estimate the prevalence (number of cases during a particular period or at a particular point in time) of DMD to be four per 100,000 individuals worldwide. Based on population figures, we estimate the number of DMD patients in the U.S. to be approximately 12,000-13,000 and in the EU, approximately 20,000. Although it is generally difficult to estimate the size of the market for pharmaceutical products, we believe that the potential market for Raxone<sup>®</sup> in DMD in the EU and the U.S. may be substantially larger than the market for Raxone<sup>®</sup> in LHON due to larger number of patients affected by DMD compared to LHON.

### c. **Treatment options and competing therapeutic approaches**

#### *Clinical benefits of Raxone<sup>®</sup>*

We believe that Raxone<sup>®</sup> improves the flow of electrons within the mitochondria, therefore increasing the energy production within cells that is impaired in DMD patients. Our phase III clinical trial with DMD patients between 10 and 18 years who were not also being treated with steroids showed, among other things, that Raxone<sup>®</sup> can slow the loss of respiratory function and to reduce bronchopulmonary complications such as ineffective cough and recurrent pulmonary infections.

#### *Steroid-based approaches*

Currently, glucocorticoids (a form of steroids) are the standard of care in the U.S. and the EU and are prescribed in order to slow the decline in muscle strength and function caused by DMD regardless of the genetic mutation underlying DMD. However, the long-term use of steroids is hindered by their well-known side effects profile, which prevents the long-term use of steroids. A recent third-party study showed that up to 42% of DMD patients aged 10 years or older had either never been treated with steroids or have discontinued their use. Loss of respiratory function with increasing age continues to be a major cause of illness and death in patients with DMD whether or not they are treated with steroids.

The current mainstay of medical therapy are prednisone and deflazacort. Prednisone received market authorization for the treatment of rheumatoid arthritis in the EU and the U.S. in 2010 and 2012, respectively, and is widely used off-label by DMD patients. EMFLAZA<sup>™</sup>, whose active ingredient is deflazacort, is, to our knowledge, the first and only steroid that is explicitly approved for the treatment of DMD, but only in the U.S. In February 2017, the FDA granted marketing authorization to Marathon Pharmaceuticals, LLC, Northbrook, Illinois, USA (“**Marathon Pharmaceuticals**”) for EMFLAZA<sup>™</sup> to treat DMD in patients five years of age or older in the U.S.. In April 2017, Marathon Pharmaceuticals sold its rights to EMFLAZA<sup>™</sup> to PTC Therapeutics, Inc., South Plainfield, New Jersey, USA (“**PTC Therapeutics**”). EMFLAZA<sup>™</sup> is currently marketed in the U.S. by PTC Therapeutics.

Deflazacort is an oxazoline derivative of prednisolone with a smaller impact on calcium metabolism. Therefore, deflazacort shows a lower risk of osteoporosis and growth rate retardation. In the EU physicians still prescribe it on an off-label basis to treat DMD.

A larger study comparing deflazacort to prednisone, carried out in Europe, showed that the two medications were similarly or equally effective in slowing the decline of muscle strength in DMD. Another European multicenter, double-blind, randomized trial of deflazacort versus prednisone in DMD showed equal efficacy in improving motor function and functional performance. (Title: Dystrophinopathies GeneReview – Corticosteroid Therapy Authors: Darras BT, Urion DK, Ghosh PS Date: April 2018.)

However, both drugs, prednisone and deflazacort, are associated with significant side effects, including obesity, delayed puberty, hypertension, cataracts, glaucoma, and emotional lability.

### ***Non-steroid based approaches***

To our knowledge, two treatments for DMD developed by third parties that are not based on steroids have been approved to date. The most advanced approaches to treat DMD are directed against the genetic cause of the disease and typically address a subset of patients.

In most of the DMD cases, the disease is caused by deletions in the gene that encodes dystrophin in the cell nucleus. EXONDYS 51™ (eteplirsen), an oligonucleotide drug (i.e., a short chain of nucleotides) developed by Sarepta Therapeutics, Cambridge (Massachusetts), USA (Sarepta), targets a subgroup of these patients who have a mutation that is amenable to so-called exon 51 skipping. Clinical trials by Sarepta have shown that eteplirsen may allow the expression of a truncated version of dystrophin. Studies by third parties estimate that eteplirsen has the potential to benefit 13% of patients with DMD. Sarepta has been marketing EXONDYS 51™ in the U.S. since 2016. Sarepta has also filed an MAA for EXONDYS 51™ with the EMA in Europe, on which it received a negative opinion from the CHMP in May 2018 and a negative decision on the re-examination procedure in September 2018.

So-called nonsense point mutations account for an additional 13% of DMD cases. These mutations in the gene that encodes dystrophin lead to a premature stop signal that prevents the complete translation of the affected gene and thus the expression of dystrophin. This group of mutations is targeted by Translarna™, a small molecule drug developed by PTC Therapeutics that it believes to increase readthrough at the premature stop signals to enable the production of the full-length dystrophin. PTC Therapeutics obtained a conditional marketing authorization for Translarna™ for the treatment of DMD in patients with such nonsense point mutation in the EU in 2014. Such conditional marketing authorization has subsequently been renewed. However, Translarna™ is currently not approved in the U.S.

Other potential therapeutic targets involve gene replacement therapy, upregulation of compensatory proteins, reduction of the inflammatory cascade, and enhancement of muscle regeneration. These treatments are still in clinical development.

#### **d. Clinical development status**

Our clinical development program with Raxone® in DMD started with a phase II randomized, placebo-controlled trial called DELPHI.

In May 2014, we completed a phase III, double-blind, placebo-controlled clinical trial called DELOS which randomized 64 patients between 10 and 18 years who were not also being treated with steroids to receive either Raxone® (900 mg/day) or a placebo for 52 weeks. The DELOS trial evaluated the efficacy and safety of Raxone® in delaying the loss of respiratory function in the respective group of DMD patients compared to placebo. The primary endpoint of the DELOS trial was the change from baseline to week 52 in percent predicted peak expiratory flow (a normalized measure of maximum exhalation air flow). The DELOS trial met its primary endpoint, suggesting that Raxone® can slow the loss of respiratory function in the respective group of DMD patients during a period of 52 weeks compared to placebo, and its data additionally demon-

strated that it can reduce bronchopulmonary complications in such patients compared to placebo. The frequency of adverse events observed in participants of the DELOS trial who were treated with Raxone<sup>®</sup> was comparable to adverse events experienced by participants of the DELOS trial who were given a placebo.

We have supported the results of the DELOS clinical trial by a so-called comparative natural history study that showed that the decline in respiratory function observed in the DMD patients who had received a placebo in the DELOS trial corresponds to the expected decline from the natural course of the disease, whereas the course of disease observed in the DMD patients who had received Raxone<sup>®</sup> in the DELOS trial would not have been expected from the natural course of the disease.

In September 2016, we started enrolling patients in another randomized, double-blind, placebo-controlled phase III clinical trial called SIDEROS. The SIDEROS trial aims at assessing the efficacy of Raxone<sup>®</sup> in slowing the rate of decline of the respiratory function in 266 DMD patients receiving steroids. The SIDEROS trial is being conducted at 67 centers in the United States, Europe and Israel and enrolls DMD patients ten years and older with declining respiratory function who are on any stable steroid treatment scheme and irrespective of the gene mutation causing DMD and irrespective of their current ability to walk. Study participants receive either Raxone<sup>®</sup> (900 mg/day) or a placebo for 78 weeks (18 months). The primary endpoint of the SIDEROS trial is change from baseline to week 78 in forced vital capacity percent predicted (a normalized measure of maximum lung capacity). Secondary endpoints include changes from baseline in percent predicted peak expiratory flow (a normalized measure of maximum exhalation air flow), time to first 10% decline in maximum lung capacity and change from baseline in inspiratory flow reserve (the difference between normal and maximum exhalation air flow). We currently expect to offer patients who have completed the SIDEROS trial the opportunity to enroll in an open label extension study in which all participants receive Raxone<sup>®</sup>. We currently expect top line data of the SIDEROS trial to be available in 2020. If the SIDEROS trial reaches its primary endpoint and if we obtain marketing authorization for Raxone<sup>®</sup> in DMD patients who are not receiving steroids, there may be potential for a future labeling extension of Raxone<sup>®</sup> to treat DMD patients receiving steroids.

In February 2018, we launched an FDA-approved EAP named “BreatheDMD” in the U.S., through which eligible patients in the U.S. with DMD who are 10 years and older and with a specified respiratory function decline can obtain access to Raxone<sup>®</sup>, at no cost, through various research centers across the U.S. In addition to providing patients access to treatment, the EAP aims at providing additional safety, tolerability, effectiveness and quality of life data. Patients are currently being enrolled in the EAP “BreatheDMD”.

In addition, we and Parent Project Muscular Dystrophy (PPMD), a U.S. patient advocacy group, conducted what is to our knowledge the first-ever patient benefit/risk survey of patients with DMD and their caregivers. The survey showed that such DMD patients and their caregivers placed a high value on treatments for DMD that could reduce bronchopulmonary complications associated with DMD.

**e. Market exclusivity and regulatory status**

We have been granted orphan drug designation for Raxone<sup>®</sup> for the treatment of DMD in the EU and the U.S. In addition, we have a method of use patent for Raxone<sup>®</sup> in DMD for the EU, Japan and the U.S., which is expected to expire in the EU and Japan in March 2027 and in the U.S. in December 2027.

We filed an MAA with the EMA for Raxone<sup>®</sup> for slowing the decline of respiratory function in DMD patients who are currently not taking steroids; under the initial wording of the proposed indication, the decline of respiratory function must be confirmed by repeated measurements of respiratory function prior to initiation of treatment, and Raxone<sup>®</sup> could be used in patients previously treated with steroids or in patients in whom steroid treatment is not tolerated or is considered inadvisable. In our initial MAA, we proposed to conduct a post-authorization safety study (“PASS”) as post-authorization measure. We submitted our MAA as a so-called Type II variation of our existing EU marketing authorization for Raxone<sup>®</sup> in LHON and based on data from the phase II DELPHI and the phase III DELOS clinical trials.

In September 2017, the CHMP issued a negative opinion on our initially submitted MAA by majority. According to the minutes of the CHMP, the CHMP was of the opinion that the study results we provided

were insufficient to determine the benefit of Raxone<sup>®</sup> in DMD patients. Although a difference in peak expiratory flow in favor of Raxone<sup>®</sup> was observed, in the CHMP's opinion there was no clear improvement in other indicators of breathing function or in muscle strength, motor function or quality of life. The CHMP also expressed concerns about the way the study was conducted and analyzed. Therefore, the CHMP was of the opinion that the benefits of Raxone<sup>®</sup> in DMD patients did not outweigh its risks. Hence, the CHMP recommended that the initially submitted MAA be rejected.

Following receipt of such negative opinion, we requested a re-examination of the opinion by the CHMP. In our request for re-examination, we submitted an updated proposal for post-authorization measures and a clarification of the wording of the indication to the effect that Raxone<sup>®</sup> could be used for slowing the decline of respiratory function in DMD patients not using steroids (*i.e.*, patients in whom steroid treatment is no longer tolerated or is considered inadvisable) whose decline of respiratory function was confirmed by repeated measurements of respiratory function prior to initiation of treatment. In addition to a PASS, we proposed to conduct an externally controlled long-term open label study after obtaining marketing authorization as additional post-authorization measure. Following the re-examination procedure, the CHMP announced on January 26, 2018, that it maintained its negative opinion by majority, *i.e.*, it recommended that the European Commission reject our amended MAA for Raxone<sup>®</sup> in DMD. The CHMP considered that the effect of Raxone<sup>®</sup> on patients' respiratory function observed in the DELOS clinical trial could be clinically relevant if it would be maintained over several years, rather than the 52 weeks observed in the DELOS clinical trial. The CHMP also expressed concerns about the way the study was conducted and analyzed. In July 2018, Santhera announced results of a comparative analysis of the Phase III DELOS trial outcome and new data from natural history studies. This analysis showed that the treatment effect with idebenone observed in the DELOS clinical trial can be linked to a delay in the initiation of assisted ventilation by three years, which is of high clinical relevance. We and our academic partners intend to prepare for the publication of additional clinical data relating to the long-term efficacy of idebenone on respiratory function outcomes in patients with DMD in the coming months, supporting the positive data from the successful Phase III DELOS clinical trial. We plan to discuss the findings with regulators in the coming months and to include them in the regulatory dossier in preparation of MMAs for Raxone<sup>®</sup> in DMD in Europe and the U.S. in 2019.

After receiving the CHMP's negative opinion for Raxone<sup>®</sup> in DMD in January 2018, we withdrew the MAA for Raxone<sup>®</sup> in DMD patients with declining respiratory function and who are not receiving steroids, including patients who were previously treated with steroids or for whom steroid treatment is not desired, not tolerated or contraindicated, that we had filed with Swissmedic, with the intention to refile at a later stage.

In the U.S., the FDA has granted fast track designation for Raxone<sup>®</sup> in DMD in addition to a rare pediatric disease designation. The FDA's fast track process is designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need for the purpose of getting them to the patient earlier. The fast track designation will allow us to request priority review if and when filing an NDA for Raxone<sup>®</sup> in DMD. In July 2016, the FDA advised us that, if and when we file an NDA with regard to Raxone<sup>®</sup> for the treatment of DMD, we should also provide the results from the SIDEROS trial (which is focused on patients who are receiving steroids), irrespective of whether we seek marketing authorization with regard to DMD patients who are receiving steroids. The FDA had previously confirmed based on SIDEROS' protocol that the SIDEROS trial has the potential, if successful, to provide the necessary efficacy data, along with data from previous clinical trials, to support the filing of an NDA for Raxone<sup>®</sup> in DMD.

In the UK, in June 2017, the Medicines and Healthcare Products Regulatory Agency ("MHRA") issued a positive scientific opinion on Raxone<sup>®</sup> for the treatment of DMD patients from the age of 10 years with respiratory function decline who are not receiving steroids. This scientific opinion followed a designation of Raxone<sup>®</sup> for the same indication as a Promising Innovative Medicine, a status similar to a breakthrough therapy designation by the FDA. By virtue of the MHRA's scientific opinion, DMD patients are granted access to Raxone<sup>®</sup> under the UK Early Access to Medicines Scheme ("EAMS"). In September 2017, the first DMD patients were enrolled in the EAMS. To our knowledge, this constitutes the first use of Raxone<sup>®</sup>

in DMD patients outside of a clinical trial. In June 2018, the MHRA confirmed its positive scientific opinion on Raxone<sup>®</sup> under the EAMS and renewed the EAMS for a further year.

### 3. Vamorolone in DMD

In November 2018, we entered into the Option Agreement with Idorsia under which we have acquired an option to obtain an exclusive sub-license to commercialize ReveraGen's vamorolone, a non-hormonal steroid modulator developed by ReveraGen for the treatment of DMD, for all indications and in all territories, except Japan and South Korea (for which Idorsia has the right under the Option Agreement, in its sole discretion, to grant us a sublicense). We will be able to exercise this option against payment of USD 30.0 million at the latest when the data from the Phase IIB study in DMD patients are available, which is expected to be the case in 2020 according to the current development plan. As initial consideration for the acquisition of the option for the exclusive sub-license relating to ReveraGen's vamorolone, we issued the 1,000,000 Idorsia Shares to Idorsia and have agreed to pay USD 20.0 million in cash from the proceeds of this Offering. Both the issuance of the 1,000,000 Idorsia Shares to Idorsia and the payment of the USD 20.0 million in cash are non-refundable. The cash amount is intended to compensate Idorsia for having already paid USD 15 million to ReveraGen to fund the ongoing Phase IIB trial in patients with DMD. For a summary description of DMD and of its prevalence see "*—Duchenne muscular dystrophy (DMD)*" beginning on page 74.

#### a. Market opportunity

Although it is generally difficult to estimate the size of the market for pharmaceutical products, we currently estimate that vamorolone – assuming its potential to become the new standard of care for DMD will in fact be realized – has a revenue potential in DMD of up to USD 500 million. This estimate is based on proprietary models employed by us and assumes that vamorolone will have a high penetration rate, replacing the current steroids in ambulatory DMD based on an improved safety profile. We also expect that vamorolone's price is likely to exceed the pricing of current steroids (*e.g.*, weight-based USD 50,000 to USD 80,000 for Emflaza by PTC Therapeutics) due to a better product profile. In particular, assuming a prevalence of 4 DMD patients in a population of 100,000, we estimate that in the U.S. and the five major markets in the EU in the aggregate, there are approximately 25,000 potential DMD patients. We further estimate that of these potential patients, approximately 4,000 patients could and would be treated with vamorolone in the U.S. and the five major markets in the EU in the aggregate. At the price range for Emflaza, which – as set out above – we would expect vamorolone to exceed, this translates into a peak sales potential of approximately USD 500 million. Relating to the risks associated with this estimate, see risk factor "*Our future profitability may be adversely affected if our estimates regarding the size of the market for our products and product candidates are inaccurate.*" beginning on page 24.

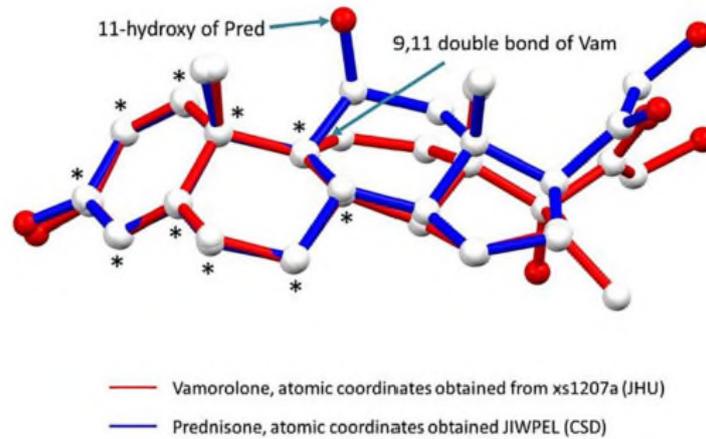
#### b. Treatment options and competing therapeutic approaches

##### *Clinical benefits of vamorolone*

Glucocorticoids while binding to their corresponding receptors regulate a plethora of physiological processes including metabolism, skeletal growth, cardiovascular activity, and immune response. They function as highly effective anti-inflammatory agents, which also suppress immunity and promote the breakdown of carbohydrates, fats and proteins. But their systemic long-term medical administration is associated with a series of significant side effects that often limit their use and detract considerably from patients' quality of life. On the molecular level glucocorticoids have many different mechanisms of action, comprising among others transcriptional repression or activation, physicochemical effects on cell membranes, cross-talk with other steroidal hormone receptors, tissue remodelling and synchronization of cell division. But each activity responsible for a desired treatment response is often related to side effects, which should not counterbalance or even exceed its positive benefit.

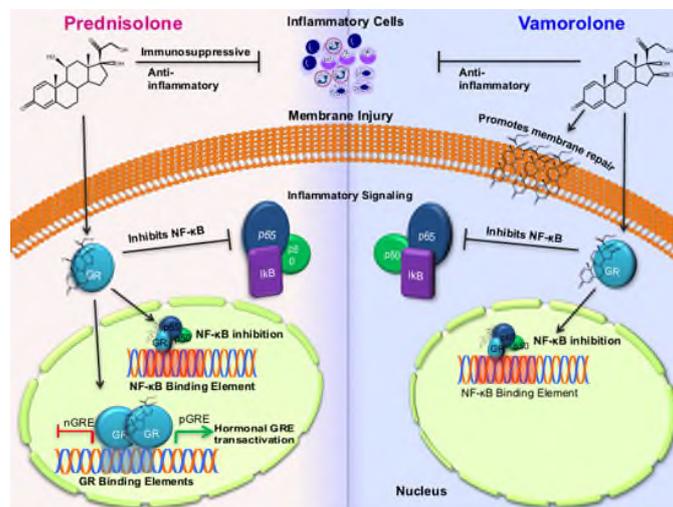
Vamorolone (VBP15) was developed as a so called dissociative steroid – chemically separating the structural properties for clinical benefit from safety concerns. It is a first-in-class multi-functional steroid that

shows potent inhibition of pro-inflammatory NFκB pathways through high-affinity binding to the glucocorticoid receptor, muscle membrane stabilization properties, and high affinity antagonism for the mineralocorticoid receptor.



Source: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6136660/pdf/nihms965660.pdf>

On the structural level vamorolone is very similar to prednisone, and they both show comparable powerful anti-inflammatory and immunosuppressive properties. But a prednisone/receptor complex couples up to form a dimer, whereas the vamorolone/receptor compound remains as a single ‘monomer’. It has been shown that these receptor/ligand monomers preferentially induce trans-repression, thus retaining anti-inflammatory properties, while minimizing transactivation. The latter is considered to be mediated by glucocorticoid/receptor dimers, which appear to be responsible for the severe side effects of glucocorticoid drugs.



Source: <http://www.reveragen.com/vamorolone/mechanism-of-action/>

Developed with these properties, vamorolone has the potential for being a replacement for long-term glucocorticoid treatment in DMD and other chronic inflammatory conditions. While retaining the anti-inflammatory efficacy via trans-repression of the NF-κB pathway and improving disease-related symptoms, vamorolone shows a significantly enhanced safety profile in multiple pre-clinical mouse models without key steroid side effects (e.g. stunted growth, hormonal imbalance, immunosuppression).

In direct comparison vamorolone has shown efficacy similar or superior to prednisone in mouse models of muscular dystrophy, lung disease, inflammatory bowel disease, and multiple sclerosis. These in vivo pre-clinical studies also showed improvement of bone safety profiles of vamorolone when benchmarked against prednisone tested in parallel, including loss of stunting of growth and osteopenia. Measures of immune

suppression were studied both in vitro and in vivo, where CD4 cells declined with prednisone, but not vamorolone. It is important to note that the potent immunosuppression effects of glucocorticoids are considered an aspect of efficacy in certain clinical indications (leukemias, and humoral autoimmunity disorders), but are considered a safety concern when prescribed in most chronic inflammatory conditions such as DMD, arthritis, asthma and others.

The first-in-human safety data obtained for vamorolone in adult volunteers agrees with and extends the pre-clinical data. It suggests a significant improvement of safety profiles relative to existing pharmacological glucocorticoid drugs. Using multiple serum biomarkers bridged to later clinical safety concerns, it has been shown that vamorolone has lost key safety concerns regarding bone turnover, insulin resistance, and immunosuppression. (Source: Steroids. 2018 June; 134: 43–52. Phase 1 trial of vamorolone, a first-in-class steroid, shows improvements in side effects via biomarkers bridged to clinical outcomes.)

Biomarker data from a phase 2a multiple ascending dose study (0.25, 0.75, 2.0 and 6.0 mg/kg/day) indicate that vamorolone has an improved safety profile compared with prednisone. This 2-week, open-label study enrolled 48 ambulant boys with DMD (4 to <7 years), with outcomes including clinical safety, pharmacokinetics, and pharmacodynamics. The biomarkers demonstrate proof-of-concept that vamorolone has an anti-inflammatory mechanism of action, and offers the potential to stabilize the myofiber membrane. In addition, bone turnover markers indicate an opposite, decreased effect on bone resorption that could reflect the novel mineralocorticoid receptor antagonism of vamorolone. (Source: Pharmacol Res. 2018 Sep 13;136:140-150. Phase IIa trial in Duchenne muscular dystrophy shows vamorolone is a first-in-class dissociative steroidal anti-inflammatory drug.)

Data from the 6-months Phase 2A open-label extension study allowed the comparison of patients treated with vamorolone to untreated patients (from natural history studies). From these data it can be concluded that vamorolone shows a dose-related increase of efficacy as seen in functional tests (such as time to stand) significantly superior to data from untreated patients. Moreover, comparison to previously collected data from patients treated with the standard glucocorticoid prednisone indicate an about 10-fold larger safety margin using validated biomarkers. Of particular relevance was the finding that unwanted weight gain can be avoided by vamorolone otherwise seen with glucocorticoids.

### ***Competing therapeutics and other treatment options***

#### **Other Steroid-based approaches**

See “—2. *Our lead product candidate: Raxone® in DMD—c. Treatment options and competing therapeutic approaches—Steroid-based approaches*” beginning on page 74.

#### **Non-steroid based approaches**

See “—2. *Our lead product candidate: Raxone® in DMD—c. Treatment options and competing therapeutic approaches—Non-Steroid-based approaches*” beginning on page 74.

### ***Clinical development***

The clinical development program of vamorolone has been subject to, and is in agreement with, scientific advice by U.S. and EU regulators.

VBP15-001 was an open-label, sequential dose-ranging study evaluating four dose levels (0.25, 0.75, 2.0, and 6.0 mg/kg/day) of vamorolone in 48 boys with DMD, 4-7 years old. The initial two-week treatment period was followed by a six-month extension at the same dose levels (clinicaltrials.gov; NCT02760264, NCT02760277). The study showed dose-related improvements of multiple function tests of strength and endurance. Clinical efficacy was demonstrated at the 2 mg/kg/day and the 6 mg/kg/day vamorolone doses compared to data from untreated patients in the CINRG Duchenne Natural History Study and was of similar magnitude to that seen in prednisone-treated patients in the historical CINRG prednisone trial. Vamorolone exhibited limited metabolic disturbance and bone turnover change at doses of 2.0 or 6.0 mg/kg/day. In

animal models of DMD, vamorolone was shown to be a dissociative steroid. It displayed fewer side effects than traditional steroids and was superior for muscle strength and membrane stabilization, two key efficacy aspects specific for DMD therapy.

VBP15-002 was a four-week, open-label, phase 2a multiple ascending dose trial, comprised of two weeks of daily treatment with vamorolone followed by a two-week washout period in steroid-naïve, 4 to <7 year old boys with DMD (NCT 02760264). Twelve patients were recruited in each of four dose groups (0.25, 0.75, 2.0 and 6.0 mg/kg/day) (48 patients total). Clinical safety through adverse event reporting was collected throughout the study. All study participants completed the four-week VBP15-002 phase 2a study and were invited to enrol in a 24-week phase 2 extension study (VBP15-003) at the same dose level, and in the long term extension study that followed (VBP15-LTE). Data have been published in *Pharmacological Research* under the title “Phase IIa trial in Duchenne muscular dystrophy shows vamorolone is a first-in-class dissociative steroidal anti-inflammatory drug”. The authors report that vamorolone was safe and well-tolerated through the highest dose tested (6.0 mg/kg/day, or about 9-times typical prednisone dose in DMD) and showed pharmacokinetics similar to prednisone.

The VBP15-003 extension was an open-label, multiple-dose study to evaluate the long-term safety, tolerability, efficacy and PD of vamorolone administered once daily by liquid oral suspension over a treatment period of 24 weeks to boys ages 4-7 years with DMD (NCT02760277). The study was completed in April 2018 and the final clinical study report is available.

The VBP15-LTE 2-year long-term extension study (NCT03038399) is still ongoing with an estimated completion date of January 2021.

VBP15-004 is a phase 2b randomized, double-blind, parallel group, placebo and active-controlled study to evaluate the efficacy, safety, PD, and population PK of vamorolone (NCT03439670). The study is recruiting patients and is planned to be completed in May 2020.

#### **c. Market exclusivity and regulatory status**

Vamorolone has been granted orphan drug designation by both the FDA and the EMA, and received fast track designation by the FDA. Upon granting of a marketing authorization in the U.S. or the EU, vamorolone would enjoy market exclusivity based on the orphan drug status by 7 years in the U.S. and 10 years in Europe from the date of the marketing authorization. The use of vamorolone as treatment of DMD is also covered by method of use patents (see “X. *The Company and Its Business—E. Additional information on our business—5. Market exclusivity and intellectual property*” beginning on page 90), providing additional and overlapping protection from generic versions until 2029.

#### **4. Our early stage pipeline**

##### **a. Omigapil as phase I product candidate in CMD**

We are exploring the compound omigapil for the treatment of CMD. We announced favorable top line data of the phase I clinical trial of omigapil in CMD (called CALLISTO) in April 2018. If the full results of this trial confirm its top line data, we will assess whether we should enter phase II of product development.

##### ***Congenital muscular dystrophy (CMD)***

CMD is a group of inherited neuromuscular conditions that causes progressive and potentially life-threatening loss of muscle tissue, affecting frequently newborns and children. CMD conditions are caused by mutations in proteins required for the muscles, and sometimes the eyes and brain, to work properly. Children born with CMD often have muscle weakness or “floppiness” and can also have stiffness of the joints, hip dislocation and a type of curvature of the spine (known as kyphoscoliosis). Affected patients have difficulties in walking, maintaining stable body posture and lifting objects. These symptoms can be present at birth or develop during childhood or later in life.

Many types of CMD are stable or progress only slowly, allowing children to acquire new skills and to live a normal lifespan. However, some severe forms of CMD cause respiratory problems that can be life-threatening as they worsen over time.

### ***Market opportunity, treatment options and competing therapeutic approaches***

The British Muscular Dystrophy Campaign estimates that two to five in 100,000 children in the UK are born with CMD.

Currently, treatment options for CMD are confined to treatment of symptoms and include physiotherapy, speech therapy, occupational therapy, respiratory support and orthopedic spine surgery. Another important aspect of disease management is supplementary nutrition to avoid malnutrition. To our knowledge, no approved pharmaceutical treatments are available or in clinical development for CMD. Also, our CALLISTO phase I clinical trial is to our knowledge the only ongoing clinical trial in CMD as of the date of this Offering Memorandum. However, we are aware of a few preclinical programs for pharmaceutical treatments of CMD.

### ***Clinical development status***

We are currently investigating omigapil as a potential treatment for CMD. Omigapil is a so-called deprenyl analogue that third-party research has shown to prevent cell death pathways (apoptosis). In preclinical research, we demonstrated that omigapil prevented apoptosis and loss of muscle tissue and that it increases body weight and survival of a disease-relevant animal model organism for CMD.

Our CALLISTO phase I clinical trial, which we conducted in collaboration with the NINDS, aimed to evaluate the pharmacokinetic profile (the absorption, metabolism and excretion of a substance in the human body), safety and tolerability of a new liquid formulation of omigapil in patients with one of two subtypes of CMD, called COL6-RD or LAMA2-RD, which together account for about up to 50% of all CMD cases. To be eligible to participate in the CALLISTO trial, patients had to be aged 5 to 16 years, be unable to walk or able to walk but with limitations at the time of screening, and had to show a clinical picture consistent with one of the two subtypes of CMD that were studied in such trial. The CALLISTO trial reached its target enrollment of 20 patients in aggregate. Top line data of the CALLISTO trial, which we announced in April 2018, suggest that the trial met its primary objective to establish a favorable pharmacokinetic profile of omigapil and demonstrated that the drug was safe and well tolerated in the patients that participated in the trial. The top line data also provide insight into the feasibility of clinical assessments (*i.e.*, potential endpoints) suitable for the design of future efficacy trials. Further development is currently being discussed with clinical experts and regulators.

In August 2016, the Office of Orphan Products Development at the FDA granted us a one-time lump-sum award of USD 246,000 in support of the CALLISTO trial. The grant was awarded through the FDA's Orphan Products Grants Program (OPGP) to support the clinical development of products for use in rare diseases where no current therapy exists. Such grants are intended for clinical studies evaluating the safety and/or effectiveness of products that could either result in, or substantially contribute to, marketing authorization of these products. The CALLISTO trial is being conducted in collaboration with the NINDS in the United States. It was previously supported by a public-private partnership comprising two patient organizations (the U.S.-based Cure CMD and the Swiss Foundation for Research on Muscle Diseases) and EndoStem, a research program funded by the European Commission's 7th Framework Program.

### ***Market exclusivity and regulatory status***

We hold method of use patents to the use of omigapil in CMD in the U.S., the EU and other jurisdiction, which will expire in 2026 or 2027, as applicable. In addition, we have a world-wide exclusive license from Novartis to develop and commercialize omigapil in CMD, including to the composition of matter patents to omigapil held by Novartis (most of which, including those in the U.S. and the EU, have, however, expired), and to know-how of Novartis in the field of CMD. In addition, we have been granted orphan drug designation for omigapil in CMD in the EU and the U.S.

In May 2016, we received fast track designation from the FDA for omigapil for the treatment of CMD. The fast track designation would allow us to request priority review from the FDA if and when we file an NDA for omigapil in CMD.

**b. POL6014 as phase I product candidate in Cystic Fibrosis**

In February 2018, we in-licensed the compound POL6014 from Polyphor. POL6014 is a selective inhibitor of an enzyme called human neutrophil elastase (“hNE”) that we believe has the potential to treat CF and other neutrophilic lung diseases (lung diseases associated with pathologically high levels of hNE) such as non-cystic fibrosis bronchiectasis (“NCFB”), alpha-1 antitrypsin deficiency (“AATD”), and primary ciliary dyskinesia (“PCD”). Based on prior development work by Polyphor, we have started a phase I MAD clinical trial (the “MAD Trial”) of POL6014 in CF in the fourth quarter of 2018.

***Cystic fibrosis (CF) and mode of action of POL6014***

CF is a rare, life-threatening, progressive genetic disease affecting primarily the lung but also the digestive system. The symptoms in the lung are characterized by build up of mucus obstructing the airways leading to persistent infection and chronic inflammation, thereby limiting the ability to breathe over time. CF is typically diagnosed in young children mostly within the first year of age.

Third-party research suggests that activated neutrophils (a type of white blood cell) liberate high levels of hNE in the lung, which in turn causes damage to structural, cellular and soluble components of the micro-environment in the lung. These high levels of hNE play a central role in the deterioration of lung function associated with CF and correlate with the severity of CF as measured by measures of lung function. We believe that inhibition of hNE may stop or slow damage to lung tissue and may help improve the overall quality of life for individuals with CF.

Pre-clinical research by Polyphor suggests that POL6014, a cyclic peptide designed by Polyphor, is a highly potent and selective inhibitor of hNE. In the phase I clinical trials conducted by Polyphor, POL6014 was administered by inhalation via an optimized eFlow<sup>®</sup> nebulizer (the “Inhaler”) developed by PARI Pharma GmbH, Gräfelting, Germany (“PARI”).

Third-party research suggests that chronic inflammation is also present in neutrophilic lung diseases other than CF such as NCFB, AATD, and PCD.

***Market opportunity, treatment options and competing therapeutic approaches***

We estimate that approximately 70,000 patients in the U.S. and the EU have CF at any point in time.

We believe that POL6014 has the potential to be applied in all CF patients, as it is not mutation-specific.

CF treatment typically consists of concomitant use of several approved medications, including CFTR (cystic fibrosis transmembrane conductance regulator) modulators (ivacaftor or ivacaftor combined with lumacaftor), mucolytics (Pulmozyme<sup>®</sup>, a drug marketed by Roche), inhaled antibiotics (tobramycin, aztreonam and azithromycin), and pancreatic enzyme products (Creon<sup>®</sup>, a drug marketed by BGP Products). Several dual and triple combination CFTR modulators are advancing in their clinical development, as are some novel gene therapy agents. These medications, however, do not significantly reduce lung inflammation, which is why we believe that there is a clinical need for drugs that target inflammation. Hence, we believe that POL6014 could also potentially be used as add-on therapy.

We are aware that CHIESI Farmaceutici S.p.A., Parma, Italy, is developing an hNE inhibitor to treat CF administered via a dry-powder inhaler (DPI) (CHF6333) and completed a phase I clinical trial in July 2017. No results have been published as of the date of this Offering Memorandum.

### *Clinical development and regulatory status and market exclusivity*

Polyphor has completed two clinical trials of POL6014 for the treatment of CF. A first-in-human single ascending dose phase I safety and tolerability trial in 48 healthy volunteers and a single ascending dose phase I safety and tolerability trial in 24 CF patients have shown that POL6014 was well tolerated and safe. The trial in CF patients also showed that levels of concentration of POL6014 in the sputum were around 1,000 fold higher than in the plasma and that POL6014 strongly inhibited hNE in the sputum tested 3 hours after administration, while the inhibitory effect was lower to absent and more variable after 24 hours.

In the license agreement with Polyphor, we have undertaken to assume the global development, regulatory filings and commercialization of POL6014. In a first step, we have recently started the multiple ascending dose (MAD) Trial which was previously planned by Polyphor. In parallel, we continue to collect preclinical safety data that, together with the results of the MAD Trial, may allow us to start a phase II efficacy trial of POL6014 in CF in 2019.

We also plan to initiate discussions with the EMA and the FDA around the development program for POL6014 in CF and potentially other indications.

Polyphor received financial support for its development program from Cystic Fibrosis Foundation Therapeutics Inc., Bethesda, Maryland, USA (“CFFT”), and we are entitled to financial support from CFFT in the form of milestone payments relating to the MAD Trial of up to USD 900,000 in aggregate.

We have a world-wide exclusive license or sublicense, as applicable, from Polyphor for the use of POL6014 in any indication. In addition, we have an exclusive world-wide sublicense from Polyphor to use the Inhaler to administer POL6014.

#### **E. Additional information on our business**

##### **1. Material agreements**

For a description of certain key risks associated with our contractual relationships see the risk factors described elsewhere in this Offering Memorandum.

##### **a. License and collaboration agreements**

###### *Collaboration and license agreement with Takeda*

In August 2013, we entered into an agreement with Takeda Pharmaceutical Company Ltd, Osaka, Japan (“**Takeda**”) by which we and Takeda terminated our previous contracts, under which Takeda had certain rights in relation to idebenone in DMD and another indication that we are no longer pursuing. In consideration of such termination, we granted Takeda the right to a low double-digit percentage of future licensing and/or sales income generated by us from idebenone in DMD, but not more than EUR 7.0 million in aggregate. In addition, we obtained the right to cross-reference Takeda’s idebenone data for regulatory use in the DMD indication and any other indication. If we make use of such cross-reference right (other than in relation to DMD and another indication), Takeda will be entitled to a low double-digit percentage of future licensing and/or sales income generated by us from the relevant products in the other indications in the amount of up to EUR 3.0 million in aggregate. Lastly, Takeda will be entitled to receive a percentage of future income generated by us from idebenone in DMD or other indications that requires a cross-reference of Takeda’s idebenone data for regulatory use, but not more than EUR 1.0 million in aggregate; such payment obligation is to offset Takeda’s waiver of a contingent claim for the same amount under an earlier agreement.

The agreement with Takeda terminates on the tenth anniversary of the first payment made by us to Takeda under the agreement. Either party may terminate the agreement in case of a material breach of the other

party. If Takeda terminates the agreement for cause, all licenses granted to us thereunder would be terminated.

#### ***Agreement with the Catholic University of Leuven***

In March 2005, we entered into an agreement with Katholieke Universiteit Leuven, Leuven, Belgium (“**KU Leuven**”), under which KU Leuven assigned its patents and patent applications relating to the use of idebenone to treat various forms of muscular dystrophy-related diseases, including DMD, to us. KU Leuven will be entitled to a success fee of up to EUR 0.4 million in aggregate (refundable from the royalty payments of KU Leuven, as described below) if and when we obtain marketing authorization for any product covered by a patent or patent application that is the subject of the agreement in a major market, which includes the EU (including the UK), the U.S., Canada or Japan. In addition, if we commercialize any such product ourselves, KU Leuven will be entitled to 5% royalties on net sales generated by us from the product. If we grant commercialization rights with regard to any such product to a third party, KU Leuven would be entitled to 15% of the consideration received by us from such third party.

The agreement with KU Leuven will terminate upon expiration of the last to expire patent that is the subject of the agreement or, if later, 10 years after the first commercial sale of any product covered by a patent or patent application that is the subject of the agreement.

#### ***License agreement with Novartis***

In June 2007, we entered into a license agreement with Novartis Pharma AG, Basel, Switzerland (“**Novartis**”), under which we in-licensed omigapil for treatment of CMD on an exclusive world-wide basis, including the composition of matter patents to omigapil held by Novartis (most of which, including those in the U.S. and the EU, however, have expired) and its know-how relating to omigapil in CMD. We undertook to use commercially reasonable efforts to develop omigapil in CMD world-wide according to a defined development plan, to seek marketing authorization therefor and to commercialize and market the respective product, if and when approved. In addition to an upfront consideration that we paid after signing of the agreement, we are required to make milestone payments to Novartis (up to a single-digit million USD figure) upon the start of a clinical trial that is intended to result in an MAA or an NDA for omigapil in CMD, upon regulatory approval for such product and after reaching certain sales levels of such product in the first major market country in the EU and in the U.S. We are also required to pay royalties to Novartis on a jurisdiction-by-jurisdiction basis, calculated on the basis of a medium to high single-digit percentage of our net sales from such product. In jurisdictions where the sale of such product does or will not make use of the relevant patent protection licensed under this agreement, we are required to pay Novartis a knowhow royalty of 50% of the royalty payments for the patent license as set out above.

Novartis has a one-time call back option to terminate the license granted to us and to obtain an exclusive world-wide license with regard to intellectual property in relation to omigapil in CMD generated by us. Such call back option becomes exercisable after completion of a clinical trial that is intended to result in an MAA or an NDA. If Novartis exercises its call back option, we would have a right to a one-off consideration as well as development cost payments and royalty payments based on Novartis’ net sales from omigapil in CMD.

The agreement with Novartis is due to terminate upon expiration of all royalty obligations that it provides. Novartis has a right to terminate the agreement for material breach or if we breach our development and commercialization obligations, and, under certain conditions, we also have the right to terminate the agreement early. In these cases, the license granted to us would terminate and we would be required to grant Novartis a worldwide, royalty-free, perpetual, non-exclusive license to use any intellectual property in relation to omigapil in CMD generated by us. In addition, Novartis may terminate the agreement if we are subject to a change of control where the acquirer owns or has in-licensed a product that is substitutable with omigapil in CMD; upon such termination, all licenses under the agreement would terminate.

### ***License agreement with Polyphor***

In February 2018, we entered into a license agreement with Polyphor Ltd, Allschwil, Switzerland (“**Polyphor**”), under which we in-licensed POL6014 in any indication on an exclusive world-wide basis in any indication (except in relation to the sub-licensed technology produced by PARI, which we sub-license in relation to the pulmonary delivery of certain liquid formulations of POL6014 via the Inhaler). Such license and sublicense (as applicable) extends to, among other things, the patents and patent applications with regard to POL6014 held by Polyphor and a licensor of Polyphor, respectively, as well as the patents licensed or sublicensed to Polyphor by PARI with regard to the use of the Inhaler to administer POL6014. We made an initial payment of CHF 6.5 million, payable and paid in 238,924 Shares, as upfront payment and for the purchase of Polyphor’s inventory of active pharmaceutical ingredient and some finished drug product containing POL6014. In addition, we agreed to cash payments of up to CHF 121 million contingent on future development, regulatory and sales milestones in relation to the milestones achieved for the first indication. We have agreed to make additional milestone payments if milestones for additional indications are reached. In addition, Polyphor is entitled to tiered royalty payments at a medium to high single-digit percentage of future net sales of POL6014-based products.

We have undertaken to use commercially reasonable efforts to develop and commercialize at least one POL6014-based product throughout the U.S., Germany, France, the UK, Italy and Spain. The agreement will terminate upon the later of the expiry of the last to expire patent that is owned and licensed by Polyphor, the twelfth anniversary of the first commercial sale of the last licensed product, and the expiration of regulatory exclusivity of the last licensed product. Either party has the right to terminate the agreement for material breach. Polyphor also has the right to terminate the license agreement if we fail to initiate the MAD trial by December 31, 2018 (such trial has now been initiated), if it is not feasible, or if we fail, to initiate the first clinical trial that is intended to result in an MAA or an NDA for POL6014 by June 30, 2021, or if certain other events occur. We have the right to terminate the license agreement with or without cause at any time with 180 days prior written notice, in which case we are required to comply with certain termination obligations unless the agreement is terminated due to a material breach of the agreement by Polyphor or an insolvency event experienced by Polyphor.

Polyphor has also transferred and assigned to us all of its current and future rights and obligations (with the exception of an obligation to make certain payments, which remains the obligation of Polyphor) under an award between the Cystic Fibrosis Foundation Therapeutics, Inc. and Polyphor regarding a clinical trial for POL6014.

### ***Option Agreement with Idorsia***

On November 20, 2018, we entered into an agreement (the “**Option Agreement**”) with Idorsia, under which we have acquired an option to obtain from Idorsia an exclusive sub-license to develop and commercialize (but not manufacture) vamorolone, a non-hormonal steroid modulator for the treatment of DMD developed by ReveraGen. Vamorolone is being developed by ReveraGen, which holds certain method of use patents for vamorolone; see “X. *The Company and Its Business—E. Additional information on our business—5. Market exclusivity and intellectual property*” beginning on page 90. ReveraGen has granted to Idorsia a worldwide, exclusive, sub-licensable option to license the product for commercialization under a collaborative agreement that was entered into in April 2016 between ReveraGen and Actelion Pharmaceuticals, Inc. and which was assigned by Actelion to Idorsia in 2017 (the “**ReveraGen Agreement**”). Idorsia has granted an option to sub-license vamorolone to us pursuant to the Option Agreement. Additionally, Idorsia will transfer know-how relevant for the development and commercialization of vamorolone as well as material and know-how for the drug production process to us. As a result, after any exercise of the option, we would become the sub-licensee of ReveraGen. Under the Option Agreement, we have acquired an option to obtain from Idorsia an exclusive, royalty-bearing, non-sublicensable sub-license to develop and commercialize (but not manufacture) vamorolone for all indications and in all territories, except Japan and South Korea. We will be able to exercise this option at the latest when the data from the Phase IIb study in DMD patients are available, which is expected to be the case in 2020 according to the current development plan. Idorsia has the right under the Option Agreement, in its sole discretion, to grant us a sublicense for Japan and South Korea. As initial consideration for the acquisition of the option for the exclusive sub-

license relating to ReveraGen's vamorolone, we issued the 1,000,000 Idorsia Shares to Idorsia and have agreed to pay USD 20.0 million in cash. Both the issuance of the 1,000,000 Idorsia Shares to Idorsia and the payment of the USD 20.0 million in cash are non-refundable. The cash consideration of USD 20 million shall be paid at the earlier of (i) within three (3) business days of the resolution of the Company's board of directors on the ascertainment and the execution of the capital increase regarding the Offered Shares, or (ii) within forty (40) days following November 20, 2018. If such payment is not made by Santhera within the applicable timeframe, Idorsia may terminate the Option Agreement with immediate effect and shall have the right to decide in its sole discretion on the enforcement of the USD 20.0 million payment. The cash payment amount is intended to compensate Idorsia for having already paid USD 15.0 million to ReveraGen to fund the ongoing Phase IIb trial in patients with DMD. The 1,000,000 Idorsia Shares came from authorized capital and were issued on November 21, 2018. We intend to pay the USD 20.0 million cash component of the consideration from the net proceeds of the sale of the Offered Shares. Under the Option Agreement, Idorsia will be entitled to receive a cash payment from us of USD 30.0 million upon exercise of the option and commercial milestone payments of up to USD 80 million in the DMD indication (up to USD 95 million in the DMD indication if a sub-license is also granted for Japan and South Korea) and four one-time sales milestone payments of up to USD 130 million in aggregate. Additional regulatory milestone payments payable by us to Idorsia for three additional indications amount to up to USD 205 million (up to USD 235 million if a sub-license is also granted for Japan) in aggregate. Upon commercialization of vamorolone, the Company has committed to pay to Idorsia tiered royalties ranging from a single-digit percentage to low double-digit percentage on the annual net sales of vamorolone. We have undertaken to use commercially reasonable efforts to further develop and, when appropriate, seek regulatory approval for the marketing and sale of vamorolone for DMD and other additional indications. Any intellectual property right generated by us under the agreement will be exclusively owned by Idorsia.

Unless the Option Agreement terminates due to expiry at the end of the option period, the agreement is due to terminate on a product-by-product and country-by-country basis upon expiration of all royalty or other payment obligations. Idorsia has the right to terminate the Option Agreement if our net equity has been reduced to less than 50% of our share capital plus legal reserves, if the ReveraGen Agreement expires or is terminated without Idorsia retaining the commercial license it obtains when exercising its option under the ReveraGen Agreement (Idorsia not being allowed to terminate the ReveraGen Agreement for convenience without our prior written consent), if we fail to obtain regulatory approval in the U.S. for the sale of vamorolone for the DMD indication within two years of the grant of the commercial license by ReveraGen to Idorsia, or if certain other events occur. We have the right to terminate the Option Agreement with or without cause at any time with 90 days prior written notice.

ReveraGen granted its consent to the execution of the Option Agreement on November 20, 2018.

**b. Distribution agreements**

In November 2016, we entered into an exclusive distribution arrangement with Ewopharma AG, Schaffhausen, Switzerland ("**Ewopharma**"), to launch Raxone<sup>®</sup> for the treatment of LHON and, if and when we receive marketing authorization, for the treatment of DMD as well, in eleven countries in Eastern Europe (Bulgaria, Croatia, Czech Republic, Hungary, Poland, Romania, Slovakia and Slovenia) and the Baltics (Estonia, Latvia and Lithuania). Ewopharma has a right to negotiate the inclusion of certain other Eastern European countries in the territory covered by the agreement if we decide to launch Raxone<sup>®</sup> in such countries. In March 2017, we entered into an exclusive distribution agreement with Pharmathen Hellas S.A., Athens, Greece ("**Pharmathen**"), to launch Raxone<sup>®</sup> for the treatment of LHON in Greece and Cyprus. In the same year, we entered into an exclusive distribution agreement with Megapharm Ltd., Raanana, Israel ("**Megapharm**"), to launch Raxone<sup>®</sup> for the treatment of LHON in Israel and the Palestinian Authority. Megapharm formally holds the marketing authorization for such product in Israel and will be required to transfer such marketing authorization to us upon termination or expiry of the distribution agreement.

In addition, we have entered into exclusive distribution agreements with other distributors with regard to other jurisdictions.

**c. Clinical development, manufacturing and other contracts**

As part of our ordinary course of business, we have entered into several contracts with third-party service providers for, *e.g.*, clinical or technical development services and the manufacturing of active pharmaceutical ingredients and finished drug products. Payments to our service providers make up a significant portion of our development expenses.

**d. CHF 60 million Senior Unsecured Convertible Bonds 2017-2022**

On February 17, 2017, the Company issued an aggregate of CHF 60 million Senior Unsecured Convertible Bonds 2017-2022, which are listed on the SIX Swiss Exchange (ISIN CH0353955195, Ticker Symbol SAN17) and governed by Swiss law (the “**Bonds**”). The nominal value of each Bond is CHF 5,000.

The Bonds carry interest at 5% *per annum* payable semi-annually on February 17 and August 17. The Bonds are senior and unsecured obligations of the Company and rank *pari passu* with all its other non-subordinated debt and will become due for redemption on February 17, 2022, unless previously converted, redeemed or purchased and canceled under their terms and conditions. We may repurchase the Bonds at any time and may also have the repurchased Bonds canceled. Out of the net proceeds of the Bonds, an amount corresponding to the interest payable on the Bonds for the first three years of their term has been put into escrow to be used for such interest payments.

The Bonds are convertible between May 17, 2017 and their date of maturity. The initial conversion price was CHF 86.4006 (corresponding to a 20% initial conversion premium over a reference share price of CHF 71.9969). However, as provided by the terms and conditions of the Bonds, the conversion price was adjusted to CHF 64.80 in February 2018. The prevailing conversion price is subject to anti-dilution and other adjustments in certain events, as further set forth in the terms and conditions of the Bonds.

The Company has the right to redeem all, but not only some of the outstanding Bonds at their principal amount, plus accrued interest, if any, at any time on or after February 17, 2019, if the volume-weighted average price of the Shares is at least 160% of the prevailing conversion price on at least 20 out of 30 consecutive trading days ending not earlier than 5 trading days prior to the Company’s redemption notice.

If a person or persons acting in concert acquire more than 50% of all voting rights (whether exercisable or not) of the Company, via a public tender offer, a merger or otherwise, or if the legal or beneficial ownership of all or substantially all of the assets of the Group is acquired by a third party, the holders of the Bond have the right to convert their Bonds at an increased conversion price or to seek redemption of their Bonds, each during a specified period and all as further specified in the terms and conditions of the Bonds.

The terms and conditions of the Bonds provide for, among other things, a negative pledge with regard to (i) marketable debt instruments and (ii) financial indebtedness exceeding CHF 10 million in the aggregate, subject to certain exceptions. In addition, the Bonds may be declared immediately due and payable in certain events of default, such as (i) a default with regard to payments or the delivery of Shares under the Bonds (subject to grace periods); (ii) material breaches of the terms and conditions of the Bonds which are not remedied in a timely manner; (iii) a cross-default with respect to financial indebtedness whose nominal value is at least CHF 5 million in the aggregate; (iv) if the Company or a material subsidiary is insolvent, bankrupt, unable to pay its debt, stops or suspends payment of material debt, proposes or applies for a stay of execution, enters into a postponement of payments, a general assignment or arrangement or composition with creditors, or a moratorium or postponement of payments is declared; (v) if the Company or a material subsidiary alters its commercial structure through bankruptcy, liquidation or disposal of all assets, the Company changes its objects or commercial activities or merges with a third party, if such action has or may have a material adverse effect on the Company’s capacity to meet its obligations in connection with the Bonds; or (vi) a dissolution of the Company or a the Company’s merger into another company where the surviving company does not assume all of the Company’s liabilities.

## **2. Distribution and marketing**

### **a. Overview**

Raxone<sup>®</sup> in LHON and our product candidates (if approved) will have different prescriber bases: primarily ophthalmologists in the case of Raxone<sup>®</sup> in LHON, primarily neurologists in the case of Raxone<sup>®</sup> in DMD and omigapil in CMD, and primarily pulmonologists in the case of POL6014 in CF. Due to limited synergy potential for marketing and sales, we may have to build separate sales channels for each of our products.

We currently sell Raxone<sup>®</sup> in LHON in more than 20 countries, either through our own subsidiaries or through a distribution partnership. Since January 2015, we have been building up a small internal sales and marketing force and expanded our operations across Europe. Our sales are primarily in Germany and France. If and when marketing authorization for Raxone<sup>®</sup> in DMD in the EU is granted, we will consider expanding our sales force and may also develop further in-house marketing, sales and distribution capabilities to commercialize the product. We may also engage additional third parties to perform these services.

We are marketing Raxone<sup>®</sup> in LHON directly to prescribers and engage in direct patient communications. We envision a similar marketing approach for Raxone<sup>®</sup> in DMD, if and when we obtain marketing authorization.

In March 2018, we decided to reorganize the management of our commercial operations. Consequently, Giovanni Stropoli stepped down from his role as Chief Commercial Officer Europe & Rest of the World and his role as a member of the Executive Management.

### **b. Presence in Europe**

We distribute Raxone<sup>®</sup> in LHON via subsidiaries in four regional country clusters: (i) the Central Europe cluster, which includes Germany, Austria and Switzerland, and is serviced via our subsidiary in Munich, Germany; (ii) the Western Europe cluster, which includes France, Spain, Portugal, the Netherlands, Belgium, Luxembourg and is serviced via our subsidiary in Nieuwegein/Utrecht, the Netherlands; (iii) the Southern Europe cluster, which includes Italy and Greece and is serviced via our subsidiary in Milan, Italy; and (iv) the Northern Europe cluster, which includes Denmark, Norway, Sweden, Finland, the UK, Ireland and Iceland and is serviced via our subsidiary in London, UK.

In other regions, we distribute Raxone<sup>®</sup> in LHON via distributors. In January 2016, we entered into a distribution and supply arrangement with Ewopharma to launch Raxone<sup>®</sup> for the treatment of LHON in eleven countries in Eastern Europe (Bulgaria, Croatia, Czech Republic, Hungary, Poland, Romania, Slovakia and Slovenia) and the Baltics (Estonia, Latvia and Lithuania) on an exclusive basis. In March 2017, we entered into an exclusive distribution agreement with Pharmathen to launch Raxone<sup>®</sup> for the treatment of LHON in Greece and Cyprus. In the same year, we entered into an exclusive distribution agreement with Megapharm to launch Raxone<sup>®</sup> for the treatment of LHON in Israel. See “—*Distribution agreements*” beginning on page 87.

### **c. Presence in the United States**

We have a U.S. subsidiary in the Boston metropolitan area. Our U.S. team currently manages our patient advocacy interactions, prepares for market entry in the U.S. and is the source of our U.S. regulatory and medical affairs expertise. A full roll-out of U.S. marketing and sales activities will only be possible upon receipt of a marketing authorization of Raxone<sup>®</sup> in DMD from the FDA.

## **3. Supply and manufacturing**

We have no internal manufacturing capabilities and rely on third parties for the manufacture, formulation, packaging, storage and distribution of our product and product candidates. See also risk factor “*We have*

*no manufacturing capabilities or capacity of our own and rely on third parties for production of Raxone<sup>®</sup> and our other compounds, omigapil and POL6014.*” beginning on page 29.

#### **4. Competition**

The pharmaceutical industry is rapidly evolving and highly competitive. Companies can expect to face significant competition from biotechnology and pharmaceutical companies, in particular when “first-in-class” products are introduced and new markets are opened. After the innovator company has successfully developed an underserved market by creating awareness of a new therapeutic agent, other companies are quick to introduce competitive products. Competition generally comes from new and existing therapies developed and marketed by large and small pharmaceutical and biotechnology companies. It is the nature of the competitive landscape that a marketer of a product has difficulty in predicting the future basis upon which it will compete with new products marketed by others.

We believe that the number of competitors currently serving or planning to enter the markets in which we are active is relatively low compared to other clinical areas.

We believe that companies that are currently developing new products for the treatment of LHON (which may compete with our own product, Raxone<sup>®</sup>) may be granted marketing authorization therefor during the next several years. Also, to our knowledge, two treatments for DMD developed by third parties that are not based on steroids have been approved to date, and there are a number of phase II clinical trials of drugs targeting muscle weakness in DMD. For overviews of therapeutic approaches that compete or may compete with our own product and product candidates see “—*Our marketed product: Raxone<sup>®</sup> in LHON—Market opportunity*” and “—*Competing therapeutic approaches*” beginning on page 70 and 73, respectively, “—*Our lead product candidate: Raxone<sup>®</sup> in DMD—Treatment options and competing therapeutic approaches*” beginning on page 74, “—*Omigapil as phase I product candidate in CMD—Market opportunity, treatment options and competing therapeutic approaches*” beginning on page 82, and “—*POL6014 as phase I product candidate in Cystic Fibrosis—Market opportunity, treatment options and competing therapeutic approaches*” beginning on page 83.

In addition to alternative therapeutic approaches, we face competition by off-label uses of idebenone (the active ingredient in Raxone<sup>®</sup>): A considerable number of physicians in Europe, and to a lesser degree in the U.S. and other countries, have been prescribing or recommending idebenone to their patients on an off-label basis. The substance is either acquired from internet sources or countries where idebenone is already approved and marketed for other indications. For instance, Takeda’s Mnesis<sup>®</sup>, 45mg tablets containing idebenone, which is registered in Italy for the treatment of “cognitive-behavioral deficits resulting from cerebral pathologies whether from vascular or degenerative origin”, is used off-label and prescribed as an unlicensed medicine in indications other than the approved one in Italy and in certain other countries. In Sweden, certain individual patients have been granted licenses for the prescription and reimbursement of Mnesis<sup>®</sup> for the treatment of LHON. For more information see risk factor “*Off-label and unlicensed uses of currently available forms of idebenone may adversely affect our sales of Raxone<sup>®</sup>.*” beginning on page 21.

Pharmacies have been compounding idebenone, *i.e.*, they made a so-called pharmacy or magistral preparation of it. We are aware that some pharmacies in Germany and the Netherlands have advertised compounded idebenone on the internet at considerably lower prices for the treatment of LHON, DMD and other indications, sometimes making reference to our clinical trials. For more information see risk factor “*Pharmacies have been compounding idebenone. Future compounding may adversely affect our sales of Raxone<sup>®</sup>.*” beginning on page 24.

#### **5. Market exclusivity and intellectual property**

Our success depends in part on our ability to obtain various layers of protection for market exclusivity through a combination of orphan drug, patent protection and documents/files protection, as available.

It is our strategy to develop and commercialize product candidates in indications qualifying for orphan drug designation in order to obtain marketing exclusivity. Orphan drug designations are available in some jurisdictions, including the U.S. and the EU, for drugs with relatively small patient populations. Generally, if a product candidate with an orphan drug designation in a particular indication subsequently receives the first marketing authorization, then the product is entitled to a period of marketing exclusivity, *i.e.*, no other marketing authorizations will be granted for the same drug for the same indication during the exclusivity period. The applicable period is 7 years in the U.S. and 10 years in the EU (to be reduced to 6 years if a drug no longer meets the criteria or is sufficiently profitable) after receipt of marketing authorization. Orphan drug exclusivity may be lost if the respective regulatory authority determines that the request for designation was materially defective, if the manufacturer is unable to assure sufficient quantity of the drug to meet patient needs, or for other reasons. To date, we have obtained orphan drug designations (a) for our marketed product, Raxone<sup>®</sup> in LHON, in the EU (maximum duration until fall 2025), the U.S. and South Korea; (b) for our product candidate Raxone<sup>®</sup> in DMD, in the EU and the U.S.; (c) for our product candidate omigapil in CMD, in the EU and the U.S.; and (d) for POL6014 in AATD, PCD and CF in the EU. We plan to seek orphan drug designation for POL6014 in CF in the U.S. as well. Vamorolone has received orphan drug designation in the U.S. and in the EU.

With respect to patent protection, we are, depending on availability and strategic importance, pursuing patents for composition of matter and method of use patents as well as new formulations for drug production. We have in-licensed omigapil in CMD as well as POL6014, and may in-license other patent-protected product candidates in the future. See “—*License and collaboration agreements*” beginning on page 84, for more information on our current licensing arrangements.

The composition of matter patents of our lead compound, idebenone (the active ingredient in Raxone<sup>®</sup>), have expired. Therefore, we can only seek method of use patent protection. Our marketed product, Raxone<sup>®</sup> in LHON, is not patent protected. For the use of Raxone<sup>®</sup> in DMD, we have been granted method of use patents that will expire in March 2026 in the EU, Japan and the U.S.

The composition of matter patents of our second compound, omigapil, which we have in-licensed from Novartis on an exclusive basis for developing and commercializing omigapil in CMD, have expired in most jurisdictions, including the U.S. and the EU. We hold method of use patents to the use of omigapil in CMD in the U.S., the EU and other jurisdictions, which will expire in 2026 or 2027, as applicable.

Polyphor holds composition of matter patents with respect to our third compound, POL6014, which we have in-licensed and sublicensed, as applicable, from Polyphor on an exclusive basis, in the EU (expiring in 2025, with potential market protection until 2030 based on a supplementary protection certificate for 5 years after launch of commercial sale in the EU) and the U.S. (expiring in 2025, with the potential of a patent term adjustment for several years after launch of commercial sale in the U.S.).

We have acquired an exclusive option to obtain from Idorsia an exclusive sub-license to commercialize ReveraGen’s vamorolone compound, including method of use patents in the U.S., the EU, China and other jurisdictions. The main patents for the use of vamorolone for the treatment of muscular dystrophy are due to expire in 2029. Because the invention was made with support from the U.S. Army Medical Research and Materiel Command (USAMRMC) as well as the National Institutes of Health (NIH), the U.S. government has reserved certain rights to vamorolone. Relatedly, ReveraGen, the inventor of vamorolone, has to comply with certain formalities, including in particular the filing of certain information with governmental databases.

We rely on trade secrets, including proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our technology and development processes that involve proprietary know-how, information or technology that is not covered by patents. In addition, we rely on our employees, advisors, third party contractors, consultants, collaboration partners as well as on CROs to develop and manufacture our product and product candidates.

We also own the rights in the trademarks SANTHERA, the Santhera logo and RAXONE, which are registered trademarks or with respect to which trademark applications are pending in the EU and certain other European countries, the U.S., Canada, and select other countries.

## 6. Employees

As of September 30, 2018, we had an aggregate of 119 employees (113.6 full time equivalent employees, “FTE”). The table below shows the number of FTE employees in different countries as of December 31, 2015, 2016 and 2017 and as of September 30, 2018:

Country	FTE (rounded) as of			
	2015	December 31, 2016	2017	September 30, 2018
Switzerland	31.5	39.6	50.9	59.5
EU	19.8	30.8	43.8	42.5
U.S.	1.0	3.0	10.5	10.6
Canada	1.0	1.0	1.0	1.0
<b>Total</b>	<b>53.3</b>	<b>74.4</b>	<b>106.2</b>	<b>113.6</b>

In addition to the above figures, we employed the equivalent of 3.0 FTEs as temporary workers as of December 31, 2017.

### Functional split (rounded) as of December 31, 2017

General & Administration, Human Resources, Legal, Finance	15%
Development & Regulatory	31%
Commercial	54%

In Switzerland, we are not subject to collective bargaining agreements or similar labor contracts. None of the Group companies has a works council. We are confident that we have good relations with our employees.

## 7. Employee benefits and pensions

All Swiss group companies provide retirement, death and disability benefits to their employees through pension plans in line with Swiss law. Until the end of 2017, the plans were operated by the AXA Foundation for Occupational Benefits, Winterthur, Switzerland. As per January 1, 2018, we changed our Swiss pension provider to PKG Pensionskasse, Lucerne, Switzerland (“PKG”). At the same time, we expanded the pension benefits of our Swiss employees with the aim to remain competitive in terms of providing benefits to our Swiss employees. As previously, the benefits under the plans that took effect as per January 1, 2018, exceed the minimum benefits required by Swiss law in some respects and are based on the accumulated savings of each employee (comprising 60% employer’s contributions, 40% employee contributions and interest credits). The amount of benefits depends on the insured compensation, age and other factors, all as set forth in the plans. In addition to the pension arrangements with PKG, we implemented a so-called “1e plan” (a pension plan enabling high-income employees to choose from different investment strategies) for our management team operated by the collective occupational benefits foundation PensFlex - Sammelstiftung für die ausserobligatorische berufliche Vorsorge, Lucerne, Switzerland. The costs of this plan are split between employer (60%) and employee (40%).

In Germany, employees are enrolled in the statutory pension insurance (*gesetzliche Rentenversicherung*), a state social security scheme. In addition, our German employees are enrolled in an occupational pension scheme providing for life-long pension payments as well as death benefits before retirement.

The pension plans for our employees in Switzerland are treated as defined benefit plans for accounting purposes. The pension plans for our employees in other countries are treated as defined contribution plans.

For more information on our pension arrangements, their accounting and associated contingent liabilities see the Notes to the 2017 Consolidated Financial Statements, in particular Note 21, included elsewhere in this Offering Memorandum.

## **8. Incentive compensation**

We currently have Board Share Appreciation Rights Plans (the “**BSARPs**”) and Employee Share Appreciation Rights Plans (the “**ESARPs**”) in place as well as certain types legacy plans under which no further grants will be made, namely Board Stock Option Plans (the “**BSOPs**”) and Employee Stock Option Plans (the “**ESOPs**”, together with the BSARPs, the ESARPs and the BSOPs, the “**Plans**”). We also used to have an Equity Incentive Plan, which expired in 2016 after the exercise of the last unexercised options. For additional information on the Plans and their accounting treatment see the Notes to the 2017 Consolidated Financial Statements, in particular Note 17, and the Notes to the Unaudited Interim Condensed Consolidated Financial Statements, in particular Note 13, all as included elsewhere in this Offering Memorandum.

### *Share Appreciation Rights Plans (BSARPs and ESARPs)*

We introduced the first BSARP (for the members of the Board) and ESARP (for the Executive Management, employees and consultants) as per July 1, 2016, as a replacement of the BSOPs and the ESOPs. The Board adopted a BSARP and an ESARP for 2016 and, with certain modifications, for 2017. In addition, the Board adopted a special ESARP for 2018 (the “**2018 ESARP**”). The BSARPs and ESARPs provide for the grant of share appreciation rights (“**SAR**”). SAR grants are made periodically at the discretion of the Board or as contractually agreed with employees. One third of the SARs vest on the first anniversary of grant; the remaining two thirds vest at the end of each following quarter during the second and third year after grant (8 times 1/12 of the SAR granted). Under the 2018 ESARP, vesting of SARs on each of these dates is conditional upon the Committee for Medicinal Products for Human Use (“**CHMP**”) of the European Medicines Agency (“**EMA**”) having approved Raxone<sup>®</sup> for the treatment of patients with DMD in the EU.

Under all BSARPs and ESARPs, SARs are exercisable after vesting during their entire term of 10 years from the grant date. At the end of the term, unexercised SARs expire without value. Upon exercise of one SAR, participants receive the difference between the Share price at the time of exercise and the base value (“exercise price” as defined upon grant) in Shares. Subsequently, participants may sell their Shares.

The ESARPs contain provisions according to which SARs vest upon a change of control event (as defined therein). In such a case, the Board may declare the forced exercise of all or some SARs. Upon a termination of the employment agreement for cause, the SARs are forfeited. In case of an ordinary termination of the employment, generally, all non-vested SARs are forfeited, while the already vested SARs continue to be exercisable. In case of termination of the employment by reason of retirement or disability, the SARs, irrespective of their vesting status, continue to exist. In case of termination of the employment by reason of death, the SARs, irrespective of their vesting status, may be exercised by the deceased’s heirs.

The BSARPs contain provisions according to which SARs vest upon a change of control event (as defined therein). In such a case, the Board may declare the forced exercise of all or some SARs. In case of a termination of the Board mandate, any unvested SARs vest. However, if a participant has committed a severe breach of his duties, all SARs are forfeited. The same is the case if a Board member tenders his resignation at his free will.

For more information on the outstanding SARs and planned SAR issuances see “—Options, warrants and conversion rights” in “Description of the Company’s Capital Structure and Shares” beginning on page 136.

### *Stock Option Plans (BSOPs and ESOPs)*

Before we adopted our first BSARP and ESARP in 2016, we had adopted a BSOP in each of the years 2015 and 2011 as well as an ESOP in each of the years 2015, 2010, 2008 and 2004, to provide incentives to members of the Board or members of the Executive Management, employees and consultants, respectively,

helping to ensure their commitment to the Group over the long-term. Under the BSOPs and ESOPs, stock option grants were made periodically at the discretion of the Board or as contractually agreed with employees. The BSOPs and ESOPs provide for the adjustment or cancelation of stock options upon termination of service, retirement, death, disability and certain corporate transactions. The implications of a participant's leaving the Group in good faith on his or her vested and unvested options vary between different BSOPs and ESOPs from different years. Each stock option entitles its holder to purchase one Share at an exercise price defined to be either (i) the volume-weighted average Share price in the three preceding months for Swiss employees (for Swiss Board members and employees), or (ii) the closing Share price on the SIX Swiss Exchange at the grant date. In general, 50% of the stock options vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. Stock options are exercisable after vesting during their entire term of 10 years as from the grant date. At the end of the term, unexercised stock options expire without value.

For more information on the outstanding stock options see “—Options, warrants and conversion rights” in “Description of the Company's Capital Structure and Shares” beginning on page 136.

### **Short-term incentives**

All employees of the Group, including the members of the Executive Management, are entitled to receive a short-term incentive in cash. In the case of the members of the Executive Management, the payment of such cash bonus is subject to the retrospective approval by the annual general meeting of shareholders based on a proposal made to it by the Board (see “—Say on pay” in “Board of Directors and Executive Management” beginning on page 127). Each member of the Executive Management has a contractually agreed maximum cash bonus which is expressed as a percentage of his or her base salary, ranging between 25% and 50%. To determine the cash amount to be paid out, the achievement of both corporate and individual goals (0% to 100%) is multiplied by a defined factor. For the year 2017, the Board determined the achievement of corporate goals to be 60%.

## **9. Facilities and real property**

Our headquarters are located in Pratteln near Basel, Switzerland. Our subsidiaries have their offices in Munich/Germany, Lörrach/Germany, Nieuwegein/the Netherlands, Milan/Italy, London/UK, Irún/Spain, Ruggell/Liechtenstein and Burlington, Massachusetts/U.S. We do not own any real property and all of our facilities are leased. The table below sets out the location, key functions and approximate area of our facilities (without parking spaces):

<b>Location</b>	<b>Key functions</b>	<b>Approximate area</b>
Pratteln, Switzerland	Group headquarter; development of pharmaceutical drugs, administrative functions	2,400 m <sup>2</sup>
Munich, Germany	Medical information	118 m <sup>2</sup>
Lörrach, Germany	Regulatory and development in the EU	20 m <sup>2</sup>
Nieuwegein, Netherlands	Medical information	165 m <sup>2</sup>
Milan, Italy	Medical information	185 m <sup>2</sup>
London, United Kingdom	Medical information	68 m <sup>2</sup>
Irún, Spain	Medical information	91 m <sup>2</sup>
Ruggell, Liechtenstein	Logistics, distribution	80 m <sup>2</sup>
Burlington, U.S.	Advocacy/patient liaison	240 m <sup>2</sup>

In February 2018, we moved our group headquarter and all other functions that were previously located in our former premises in Liestal, Switzerland (approximate area: 1,500 m<sup>2</sup>) to our current headquarter in Pratteln, Switzerland. In preparation for moving into our new headquarter, we spent an aggregate amount of CHF 2.0 million for leasehold improvements and office equipment purchases. The lease of our former premises in Liestal, Switzerland, terminated as of March 31, 2018.

We are currently evaluating whether to rent additional office space in Munich, Germany.

We are not aware of any environmental issues or other constraints that would materially impact the current or intended use of our facilities.

## 10. Legal and regulatory proceedings

As of the date of this Offering Memorandum, there are no pending or threatened court, arbitral or administrative proceedings that are of material importance to our business, financial condition, results of operations or prospects, other than the filings pending before medical product authorities and similar authorities discussed elsewhere in this Offering Memorandum.

## 11. Insurance

We hold group-wide insurance policies for a variety of risks and activities, in particular public and product liability, transport insurance, employment practices liability, and directors' and officers' liability. Additionally, we hold insurance policies on a local level that cover employees' social benefits according to the applicable law, local custom or our internal policies. Further, we hold local policies at regional or local level, including for certain specialized risks such as fire and business interruption in Europe and electronic data processing in Switzerland.

## 12. Subsidiaries, associated companies and joint ventures

The following table sets out the Company's subsidiaries:

Name of subsidiary	Share capital	Domicile	Activities
Santhera Pharmaceuticals (Schweiz) AG	CHF 125,000	Pratteln, Switzerland	Development of pharmaceuticals drugs, administrative functions
Santhera Pharmaceuticals (Liechtenstein) AG <sup>(1)</sup>	CHF 50,000	Ruggell, Liechtenstein	Logistics/distribution
Santhera (Germany) GmbH <sup>(1)</sup>	EUR 50,000	Munich, Germany	Medical information
Santhera (Netherlands) B.V. <sup>(1)</sup>	EUR 50,000	Nieuwegein, Netherlands	Medical information
Santhera (UK) Limited <sup>(1)</sup>	GBP 50,000	London, UK	Medical information
Santhera (Italy) S.r.l. <sup>(1)</sup>	EUR 50,000	Milan, Italy	Medical information
Santhera Pharmaceuticals (Spain), S.L. <sup>(1)</sup>	EUR 50,000	Irún, Spain	Medical information
Santhera Pharmaceuticals (Canada), Inc.	CAD 1,000	Montréal, Canada	Development of pharmaceutical drugs
Santhera Pharmaceuticals (USA), Inc.	USD 1,000	Burlington, Massachusetts, U.S.	Advocacy/patient liaison
Santhera Pharmaceuticals (Deutschland) GmbH	EUR 25,000	Lörrach, Germany	Regulatory and development in the EU
Oy Santhera Pharmaceuticals (Finland) Ltd	EUR 25,000	Helsinki, Finland	Administrative

(1) Direct subsidiary of Santhera Pharmaceuticals (Schweiz) AG.

Unless otherwise indicated, the above-listed subsidiaries are directly and wholly-owned by the Company.

The Company does not have any associated companies or joint ventures.

### 13. Investments

The following table sets out our capital expenditures for the financial years ended December 31, 2015, 2016 and 2017 and for the nine month period ended September 30, 2018:

in CHF thousands	For the financial year ended December 31,			Nine month period ended September 30, 2018
	2015	2016	2017	
Investments in tangible assets .....	(350)	(289)	(1,261)	(1,271)
Investments in intangible assets .....	(165)	(86)	(136)	(33)
<b>Total capital expenditures .....</b>	<b>(515)</b>	<b>(375)</b>	<b>(1,397)</b>	<b>(1,304)</b>

Our investments in tangible assets in the financial year ended December 31, 2017, mainly consisted of leasehold improvements for our new headquarter in Pratteln, Switzerland, which we moved into in February 2018.

In February 2018, we in-licensed the compound POL6014 from Polyphor (see “—*License and collaboration agreements—License agreement with Polyphor*” in “*The Company and its Business*” beginning on page 86). The initial payment of CHF 6.5 million, payable and paid in 238,924 newly-issued Shares was both upfront consideration for the license and the purchase price for Polyphor’s inventory of active pharmaceutical ingredient and some finished drug product containing POL6014.

We announced in March 2018 that the top line data of the phase I/II clinical trial for Raxone® in primary progressive multiple sclerosis (“**PPMS**”), a form of multiple sclerosis, that the NINDS conducted in collaboration with us, did not show a difference in the measure of disease progression used in the trial between the patients treated with Raxone® and the patients who were given placebo. Therefore, we do not expect to conduct any further trials of Raxone® in PPMS and we plan to terminate our exclusive license from the NIH for the use of idebenone in PPMS.

In November 2018, we entered into an agreement (the “**Option Agreement**”) with Idorsia under which we have acquired an option to obtain an exclusive sub-license to commercialize ReveraGen’s vamorolone, a non-hormonal steroid modulator developed by ReveraGen for the treatment of DMD, for all indications and in all territories, except Japan and South Korea (for which Idorsia has the right under the Option Agreement, in its sole discretion, to grant us a sublicense). We will be able to exercise this option against payment of USD 30.0 million at the latest when the data from the Phase IIb study in DMD patients are available, which is expected to be the case in 2020 according to the current development plan. As initial consideration for the acquisition of the option for the exclusive sub-license to ReveraGen’s vamorolone, we issued the 1,000,000 Idorsia Shares to Idorsia and have agreed to pay USD 20.0 million in cash, which we intend to fund from the net proceeds of this Offering. Both the issuance of the 1,000,000 Idorsia Shares to Idorsia and the payment of the USD 20.0 million in cash are non-refundable. The cash amount is intended to compensate Idorsia for having already paid USD 15.0 million to ReveraGen to fund the ongoing Phase IIb trial in patients with DMD. For a summary description of DMD and of its prevalence see “—*Duchenne muscular dystrophy (DMD)*” beginning on page 74.

As of the date of this Offering Memorandum, other than as disclosed in this Offering Memorandum, we are not making any material capital expenditures and have not contractually committed to make significant capital expenditures in the future.

## **XI. LEGAL AND REGULATORY ENVIRONMENT**

*The following summary is based on the laws, regulations and regulatory practices of the United States and the European Union as in effect on the date hereof, which are subject to change (or subject to changes in interpretation), possibly with retroactive effect.*

### **A. U.S. government regulation**

In the United States, the U.S. Food and Drug Administration (the “**FDA**”) regulates drugs under the Federal Food, Drug, and Cosmetic Act (the “**FDCA**”) and, in the case of therapeutic biologics (biological products), the Public Health Services Act (“**PHSA**”), and its implementing regulations. Based on FDA guidance indicating that a “chemically synthesized polypeptide” is any alpha amino acid polymer that is made entirely by chemical synthesis and is less than 100 amino acids in size, we believe that our products will not be treated as biologics. Drugs are also subject to other federal, state and local statutes and regulations. The FDA and comparable regulatory agencies in state and local jurisdictions impose substantial requirements upon, among other things, the testing, development, manufacture, quality control, safety, purity, potency, labeling, storage, distribution, record keeping and reporting, approval, import and export, advertising and promotion, and post-market surveillance of products.

The FDA’s policies may change and additional government regulations may be enacted that could prevent or delay marketing approval of any product candidates, product or manufacturing changes, additional disease indications, or label changes. The Group cannot predict the likelihood, nature or extent of government regulation that might arise from future legislative or administrative action.

Failure to comply with the applicable United States regulatory requirements at any time during the product development process, approval process or after approval may subject an applicant to administrative or judicial enforcement actions. These actions could include the suspension or termination of clinical trials by the FDA, the FDA’s refusal to approve pending applications or supplemental applications, withdrawal of an approval, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, import detention, injunctions, fines, civil penalties or criminal prosecution. Any such administrative or judicial action could have a material adverse effect on the Group.

#### **1. Drug development process**

The process required by the FDA before a drug or biologic may be marketed in the United States generally involves the following:

- the completion of preclinical laboratory tests and animal studies conducted in accordance with applicable regulations, including the FDA’s Good Laboratory Practice (“**GLP**”) regulations;
- the submission to the FDA of an Investigational New Drug (“**IND**”) application for human clinical testing, which must become effective before human clinical trials commence;
- the approval by an independent institutional review board (“**IRB**”) representing each clinical site before a clinical trial may be initiated;
- the performance of adequate and well-controlled human clinical trials in accordance with applicable regulations, including the FDA’s Good Clinical Practice (“**GCP**”) regulations, to establish the safety and efficacy of the proposed product for its intended use or uses;
- the submission to the FDA of a New Drug Application (“**NDA**”) for a drug, or a Biologics License Application (“**BLA**”) for a biologic;
- a determination by the FDA within 60 days of its receipt of an NDA or BLA to accept the NDA or BLA for filing and review;
- satisfactory completion of an FDA inspection of the manufacturing facilities at which the product is made to assess compliance with the FDA’s current Good Manufacturing Practice (“**cGMP**”)

regulations to ensure that the facilities, methods and controls are adequate to preserve the 'product's identity, strength, quality and purity;

- a potential FDA audit of the preclinical and/or clinical trial sites that generated the data in support of the NDA or BLA; and
- the FDA's review and approval of an NDA or BLA prior to any commercial marketing or sale of the product in the United States and compliance with any post-authorization requirements.

## **2. Preclinical testing**

Before testing any compounds with potential therapeutic value in humans, the product candidate enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry and formulation, as well as animal studies to assess the potential safety, toxicity profile and efficacy of the product for initial testing in humans. The conduct of the preclinical tests must comply with federal regulations and requirements, including GLP regulations.

## **3. IND application**

Prior to commencing the first clinical trial in humans, an IND application must be submitted to the FDA, and the IND application must become effective. A sponsor must submit preclinical testing results to the FDA as part of the IND application and the FDA must evaluate whether there is an adequate basis for testing the product in humans. The IND application will automatically become effective 30 days after receipt by the FDA, unless the FDA within the 30-day time period raises concerns or questions about the submitted data or the conduct of the proposed clinical trial and places the IND application on clinical hold. In this case, the IND application sponsor must resolve any outstanding concerns with the FDA before clinical trial may begin. A separate submission to the existing IND application must be made for each successive clinical trial to be conducted during product development.

## **4. Clinical trials**

Clinical trials involve the administration of the product candidates to healthy volunteers or patients with the disease to be treated under the supervision of a qualified principal investigator. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety, and the efficacy criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND application. Further, each clinical trial must be reviewed and approved by an IRB, either centrally or individually at each institution at which the clinical trial will be conducted. The IRB will consider, among other things, ethical factors, the safety of human subjects and the possible liability of the institution. Additionally, some clinical trials (typically phase III clinical trials) are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board or committee. This group provides confirmation as to whether or not a trial may move forward at designated check points based on access to certain data from the study.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and written IND safety reports must be submitted to the FDA and the investigators for serious and unexpected adverse events or any finding from tests in laboratory animals that suggests a significant risk for human subjects. There are also requirements governing the reporting of ongoing clinical trials and clinical trial results to public registries.

The FDA, the IRB or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the product has been associated with unexpected serious harm to patients. A sponsor may also suspend or terminate a clinical trial based on evolving business objectives and/or competitive climate.

Clinical trials are typically conducted in three sequential phases prior to approval, but the phases may overlap or be combined. These phases generally include the following:

Phase I. Phase I clinical trials represent the initial introduction of a product candidate into human subjects, frequently healthy volunteers. In phase I, the product candidate is usually tested for safety, including adverse effects, dosage tolerance, absorption, distribution, metabolism, excretion and pharmacodynamics. Phase I studies are sometimes separated into phase 1a and phase 1b. Although there is no specific regulatory definition, phase 1b is often used to denote studies in patients rather than healthy volunteers (in which clinical responses as well as safety are evaluated), studies conducted with combinations of investigational agents, multiple dose studies or expanded cohort studies (as opposed to single ascending dose studies) or clinical pharmacology/pharmacokinetic studies.

Phase II. Phase II clinical trials usually involve studies in a limited patient population to (1) preliminarily evaluate the efficacy of the product candidate for specific indications, (2) determine dosage tolerance, optimal dosage and dosing schedule and (3) continue to identify possible adverse effects and safety risks.

Phase III. If a product candidate is found to be potentially effective and to have an acceptable safety profile in phase II studies, the clinical trial program will be expanded to phase III clinical studies to further demonstrate clinical efficacy, optimal dosage and safety within an expanded patient population at geographically dispersed clinical study sites. These clinical trials are intended to establish the overall benefit-risk ratio of the product and to provide an adequate basis for product approval by the FDA.

A pivotal study is a clinical study that adequately meets FDA requirements for the evaluation of a product candidate's efficacy and safety such that it can be used to justify the approval of the product. Generally, pivotal studies are also phase III studies, but may be phase II studies if the trial design provides a reliable assessment of clinical benefit, particularly in situations. See also "*Expedited review programs*" beginning on page 101 below.

Post-authorization studies, or phase IV clinical trials, may be conducted after initial marketing approval. These studies may be required by the FDA as a condition of approval and are used to gain additional experience from the treatment of patients in the intended therapeutic indication. The FDA also has express statutory authority to require post-market clinical studies to address safety issues.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must include developed methods for testing the identity, strength, quality and purity of the finished product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

## **5. Disclosure of clinical trial information**

Sponsors of clinical trials (other than phase I trials) of FDA-regulated products, including drugs and biologics, are required to register and disclose certain clinical trial information. Information related to the product, comparator, patient population, phase of investigation, trial sites and investigators and other aspects of the clinical trial is made public as part of the registration. Sponsors are also obligated to disclose the results of their clinical trials after completion. Disclosure of the results of certain trials may be delayed until the new product or new indication being studied has been approved. However, there are evolving rules and increasing requirements for publication of trial-related information, and it is possible that data and other information from trials involving products that never garner approval could in the future be required to be disclosed. In addition, publication policies of major medical journals mandate certain registration and disclosures as a pre-condition for potential publication, even when this is not presently mandated as a matter of law. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

## 6. FDA NDA and BLA review and approval processes

The results of preclinical studies and clinical trials, including negative or ambiguous results as well as positive findings, together with detailed information on the manufacture, composition and quality of the product as well as the behavior of the product in the body, are submitted to the FDA in the form of an NDA for a new drug or BLA for a biologic, requesting approval to market the product. Data can come from company-sponsored clinical trials intended to test the safety and effectiveness of a use of a product, or from a number of alternative sources, including studies initiated by investigators. The FDA has substantial discretion in the approval process and may refuse to accept any application or decide that the data is insufficient for approval and require additional preclinical, clinical or other studies.

The cost of preparing and submitting an NDA or BLA is substantial. Under federal law, NDAs and BLAs are subject to substantial application user fees and the sponsor of an approved NDA or BLA is also subject to annual product and establishment user fees. Under the Prescription Drug User Fee Act (“**PDUFA**”), each NDA or BLA must be accompanied by a user fee. The FDA adjusts the PDUFA user fees on an annual basis. For federal fiscal year 2018, the submission of an NDA is subject to an application user fee of USD 2,421,495. PDUFA also imposes a program fee (USD 304,162), which is assessed annually for eligible products. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on NDAs or BLAs for products designated as orphan drugs, unless the product also includes a non-orphan indication.

In addition, under the Pediatric Research Equity Act (the “**PREA**”), an NDA or BLA or supplement to an NDA or BLA must contain data to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any product for an indication for which orphan designation has been granted. However, if only one indication for a product has orphan designation, a pediatric assessment may still be required for any applications to market that same product for the non-orphan indication(s).

Once the NDA or BLA submission has been submitted, the FDA has 60 days after submission of the NDA or BLA to conduct an initial review to determine whether it is sufficient to accept for filing. Once the submission is accepted for filing, the FDA begins an in-depth review. Under the PDUFA, the FDA sets a goal date by which it plans to complete its review. This is typically 12 months from the date of submission of the NDA or BLA application (10 months from the time at which FDA accepts the NDA or BLA application for filing). The review process is often extended by FDA requests for additional information or clarification. The FDA reviews an NDA to determine, among other things, whether a product candidate is safe and effective for its intended use and indication for use, including use of a product as a combination therapy, and a BLA to determine whether the biologic is safe, pure, and potent for its intended use. The FDA also evaluates whether the product’s manufacturing is cGMP-compliant to assure and preserve the product candidate’s identity, strength, quality and purity. Before approving an NDA or BLA, the FDA will typically inspect the facilities at which the active ingredient and the formulated product candidate are manufactured and may also inspect clinical trial sites for integrity of data supporting safety and efficacy. The FDA may also convene an advisory committee of external experts to provide input on certain review issues relating to risk, benefit and interpretation of clinical trial data. The FDA is not bound by the recommendations of an advisory committee, but generally follows such recommendations in making its decisions. The FDA may delay approval of an NDA or BLA if applicable regulatory criteria are not satisfied and/or the FDA requires additional testing or information. The FDA may require post-marketing testing and surveillance to monitor safety or efficacy of a product.

After the FDA evaluates the NDA or BLA, reviews the information that will go on the product’s professional labeling, and conducts inspections of manufacturing facilities at which the active ingredient and the formulated product candidate will be manufactured, it may issue an approval letter or a Complete Response Letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A Complete Response Letter indicates that the review cycle of the application is complete and the application is not ready for approval. A Complete Response Letter may require

additional clinical data and/or an additional pivotal phase III clinical trial(s), and/or other significant, expensive and time-consuming requirements related to clinical trials, preclinical studies or manufacturing. Even if such additional information is submitted, the FDA may ultimately decide that the NDA or BLA does not satisfy the criteria for approval. The FDA could also approve the NDA or BLA with a Risk Evaluation and Mitigation Strategy (“REMS”) plan to mitigate risks, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. The FDA also may condition approval on, among other things, changes to proposed labeling, development of adequate controls and specifications, or a commitment to conduct one or more post-market studies or clinical trials. Such post-market testing may include phase IV clinical trials and surveillance to further assess and monitor the product’s safety and effectiveness after commercialization.

## **7. Companion diagnostics**

The FDA issued a final guidance document in July 2014 addressing agency policy in relation to *in vitro* diagnostic medical devices and *in vitro* companion diagnostic tests. The guidance explains that for some drugs and therapeutic biologics, the use of a companion diagnostic test is essential for the safe and effective use of the product, such as when the use of a product is limited to a specific subpopulation that can be identified by using the test. According to the guidance, the FDA generally will not approve such a product if the companion diagnostic is not also approved or cleared for the appropriate indication, and accordingly the therapeutic product and companion diagnostic should be developed and approved or cleared contemporaneously. However, the FDA may decide that it is appropriate to approve such a product without an approved or cleared *in vitro* companion diagnostic when the drug or therapeutic biologic is intended to treat a serious or life-threatening condition for which no satisfactory alternative treatment exists and the FDA determines that the benefits from the use of a product with an unapproved or uncleared *in vitro* companion diagnostic are so pronounced as to outweigh the risks from the lack of an approved or cleared *in vitro* companion diagnostic. The FDA encourages sponsors considering developing a therapeutic product that requires a companion diagnostic to request a meeting with both relevant device and therapeutic product review divisions to ensure that the product development plan will produce sufficient data to establish the safety and effectiveness of both the therapeutic product and the companion diagnostic. Because the FDA’s policy on companion diagnostics is set forth only in guidance, this policy is subject to change and is not legally binding.

## **8. Certain U.S. regulatory incentives and other programs**

### **a. Expedited review programs**

The FDA has established four programs that are intended to facilitate and expedite development and review of new drugs and biologics to address unmet medical need in the treatment of a serious or life-threatening condition: fast track designation, breakthrough therapy designation, priority review designation and accelerated approval.

Fast track designation. The FDA has a fast track program that is intended to facilitate development and expedite review of the process for reviewing new product candidates that meet certain criteria. Specifically, new products are eligible for fast track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs for the disease or condition. Fast track designation applies to the combination of the product and the specific indication for which it is being studied. There are opportunities for frequent interactions with the review team for a fast track product candidate. These include meetings with FDA, including pre-IND meetings, end-of-phase I meetings, and end-of-phase II meetings to discuss study design, extent of safety data required to support approval, dose-response concerns, and use of biomarkers. Other meetings may be scheduled as appropriate (*e.g.*, to discuss accelerated approval, the structure and content of an NDA or BLA, and other critical issues). For a fast track product candidate, the FDA may consider for review sections of the NDA or BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA or BLA, the FDA agrees to accept sections of the NDA or BLA and

determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA or BLA. The FDA may rescind a fast track designation in the qualifying criteria for designation are no longer met. In addition, a product candidate with a fast track designation may also be eligible for a priority review designation if supported by clinical data at the time of the NDA or BLA submission.

Breakthrough therapy designation. The FDA may also expedite the review of a drug or biologic designated as a breakthrough therapy, which is a product that is intended, alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A sponsor may request the FDA to designate a product as a breakthrough therapy at the time of, or any time after, the submission of an IND application for the drug or biologic. The designation of a product as a breakthrough therapy provides the same benefits as are available under the fast track program, as well as intensive FDA guidance on the product candidate's development program. If the FDA designates a product as a breakthrough therapy, it must take actions appropriate to expedite the development and review of the NDA or BLA, which may include holding meetings with the sponsor and the review team throughout the development of the product, providing timely advice to, and interactive communication with, the sponsor regarding the development of the product to ensure that the development program to gather the nonclinical and clinical data necessary for approval is as efficient as practicable, involving senior managers and experienced review staff, as appropriate, in a collaborative, cross-disciplinary review, assigning a cross-disciplinary project lead for the FDA review team to facilitate an efficient review of the development program and to serve as a scientific liaison between the review team and the sponsor, and taking steps to ensure that the design of the clinical trials is as efficient as practicable, when scientifically appropriate, such as by minimizing the number of patients exposed to a potentially less efficacious treatment. In addition, the FDA may consider for review sections of the NDA or BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the NDA or BLA, the FDA agrees to accept sections of the NDA or BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the NDA or BLA. The FDA may rescind a breakthrough therapy designation in the future if further clinical development later shows that the criteria for designation are no longer met. In addition, a product candidate with a breakthrough therapy designation may also be eligible for a priority review designation if supported by clinical data at the time of the NDA or BLA submission.

Accelerated approval. The FDA may grant accelerated approval to a product candidate for a serious or life-threatening disease or condition upon a determination that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a product receiving accelerated approval perform adequate and well-controlled post-marketing clinical studies, with failure to complete such studies or failure to demonstrate the relevant clinical benefit potentially leading to withdrawal of the approval. In addition, the FDA requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

Priority review designation. An NDA or BLA will receive priority review designation if it is for a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions. A priority review designation is intended to direct overall attention and resources to the evaluation of such applications and means that the FDA's goal is to take action on an application within six months (compared to ten months under standard review).

Fast track designation, breakthrough therapy designation, priority review designation and accelerated approval do not change the standards for approval, but may expedite the development or review process.

**b. Special protocol assessment**

The FDA and an IND sponsor may agree in writing on the design and size of clinical studies intended to form the primary basis of a claim of effectiveness in an NDA or BLA. This process is known as a special protocol assessment (“SPA”). Upon a specific request for a SPA by an IND sponsor, the FDA will evaluate the protocol. If a SPA agreement is reached, however, it is not a guarantee of product approval by the FDA or approval of any permissible claims about the product. The FDA retains significant latitude and discretion in interpreting the terms of the SPA agreement and the data and results from any study that is the subject of the SPA agreement. In particular, the SPA agreement is not binding on the FDA if previously unrecognized public health concerns later come to light, other new scientific concerns regarding product safety or efficacy arise, the IND sponsor fails to comply with the protocol agreed upon, or the relevant data, assumptions, or information provided by the IND sponsor when requesting a SPA agreement change, are found to be false statements or misstatements, or are found to omit relevant facts. A SPA agreement may not be changed by the sponsor or the FDA after the trial begins except with the written agreement of the sponsor and the FDA, or if the FDA determines that a substantial scientific issue essential to determining the safety or effectiveness of the product was identified after the testing began.

**c. Orphan drug designation**

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug or biologic candidate intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States per year, or, if it affects more than 200,000 individuals in the United States per year, there is no reasonable expectation that cost of research and development of the drug or biologic for the indication can be recovered by sales of the drug or biologic in the United States. Applications for orphan drug designation must be submitted to the FDA Office of Orphan Products Development (“OOPD”). Orphan drug designation must be requested before submitting an NDA or BLA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Although there may be some increased communication opportunities, orphan drug designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

A sponsor may request orphan drug designation not only for a previously unapproved drug or biologic but also for a drug or biologic that has already been approved and is already being marketed for a different use. A sponsor may also file a common application for orphan drug designation in the European Union and in the United States if it wishes to receive orphan drug designation in both territories. In that case, a common application must be filed with both the European Medicines Agency (“EMA”) and the OOPD.

If a drug or biologic candidate that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications to market the same product for the same indication for seven years, except in very limited circumstances, such as if the second applicant demonstrates the clinical superiority of its product. Orphan drug exclusivity does not prevent the FDA from approving a different product for the same disease or condition, or the same product for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA or BLA application user fee. Orphan drug exclusivity could block the approval of the Group’s drug candidates for seven years if a competitor obtains approval of the same product as defined by the FDA or if the Group’s drug candidate is determined to be contained within the competitor’s product for the same indication or disease.

The Orphan Products Grants Program in the FDA’s Office of Orphan Products Development, with an annual budget of approximately USD 15 million, supports clinical development of products including drugs, biologics, medical devices and medical foods for use in rare diseases and conditions where no therapy exists or where the proposed product will be superior to the existing therapy. This program provides grants for clinical studies on safety and/or effectiveness that will either result in, or substantially contribute to, market approval of these products.

The FDA expects holders of exclusivity for orphan drugs to assure the availability of sufficient quantities of their orphan drugs to meet the needs of patients. Failure to do so could result in the withdrawal of marketing exclusivity for the orphan drug.

## 9. Hatch-Waxman Act

Under the Drug Price Competition and Patent Term Restoration Act of 1984 (the “**Hatch-Waxman Act**”), the U.S. Congress created an abbreviated FDA review process for generic versions of approved pioneer (brand name) NDA products. In considering whether to approve such a generic product submitted under an Abbreviated New Drug Application (“**ANDA**”), the FDA generally requires that an ANDA applicant demonstrate that the proposed generic drug product’s active ingredient, strength, dosage form, and route of administration are the same as that of the reference product, that the two products are bioequivalent, that any impurities in the proposed product do not affect the product’s safety or effectiveness, and that its manufacturing processes and methods ensure the consistent potency and purity of its proposed product. Similarly, section 505(b)(2) of the Federal Food, Drug, and Cosmetic Act provides a reduced burden of demonstrating safety and effectiveness for an NDA for a product that is similar, but not identical, to the pioneer product.

The Hatch Waxman Act requires NDA applicants and NDA holders to provide certain information about patents related to the product for listing in its publication Approved Drug Products with Therapeutic Equivalence Evaluations, referred to as the Orange Book. ANDA and 505(b)(2) applicants who seek to reference a pioneer product must then certify regarding each of the patents listed with the FDA for the reference product. A certification that a listed patent is invalid or will not be infringed by the marketing of the applicant’s product is called a “Paragraph IV certification.”

The Hatch Waxman Act also provides periods of regulatory exclusivity for certain pioneer products during which FDA review or approval of an ANDA or 505(b)(2) application is precluded. If the pioneer product is a New Chemical Entity (an “**NCE**”), the FDA is precluded for a period of five years from accepting for review an ANDA or 505(b)(2) application for the same chemical entity. Under NCE exclusivity, the FDA may accept an ANDA or 505(b)(2) application for review after four years, however, if that application contains a Paragraph IV certification challenging one of the pioneer’s listed patents.

The Hatch Waxman Act also provides three years of exclusivity for applications containing the results of new clinical investigations (other than bioavailability studies) essential to the FDA’s approval of new uses of approved products, such as new indications, dosage forms, strengths, or conditions of use. During this three-year exclusivity period, the FDA may review but not approve an ANDA or 505(b)(2) application for a product with the same conditions of use as supported by those new clinical investigations. This exclusivity will not necessarily prohibit the FDA from accepting or approving ANDAs or 505(b)(2) applications for other products containing the same active ingredient.

If an ANDA or 505(b)(2) application containing a Paragraph IV certification is accepted for filing by the FDA, the applicant must within 20 days provide notice to the NDA holder and patent owner that the application has been submitted and provide the factual and legal basis for the applicant’s opinion that the patent is invalid or not infringed. The NDA holder or patent owner may then file suit against the ANDA or 505(b)(2) applicant for patent infringement. If a suit is filed within 45 days of receiving notice of the Paragraph IV certification, the FDA is precluded from approving the ANDA or 505(b)(2) application for a period of 30 months. The 30-month stay generally begins on the date of the receipt of notice by the NDA holder or patent owner. If the pioneer product has NCE exclusivity and the pioneer files suit against the ANDA or 505(b)(2) application during the fifth year of exclusivity, however, the 30-month stay will not be triggered until five years from the date of the reference product’s approval. The FDA may approve the proposed product before the expiration of the 30-month stay if a court finds the patent invalid or not infringed or if the court shortens the period because the parties have failed to cooperate in expediting the litigation.

## **10. Post-authorization requirements**

Any products manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

### **a. Good Manufacturing Practices**

Manufacturers and other entities involved in the manufacture and distribution of approved drugs or biologics are required to register their facilities with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP requirements, which impose certain quality processes, manufacturing controls and documentation requirements upon manufacturers in order to ensure that the product is safe, has the identity and strength, and meets the quality and purity characteristics that it purports to have. The FDA and certain states also impose requirements on manufacturers and distributors to establish the pedigree of product in the chain of distribution, including some states that require manufacturers and others to adopt new technology capable of tracking and tracing product as it moves through the distribution chain. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Noncompliance with cGMP or other requirements can result in issuance of warning letters, civil and criminal penalties, seizures and injunctive action.

### **b. Advertising and promotion**

The FDA strictly regulates marketing, labeling, advertising and promotion of an approved product. While doctors are free to prescribe any product approved by the FDA for any use, a company can only make claims relating to safety and efficacy of a drug or biologic that are consistent with FDA approval, and the company is allowed to actively market a product only for the particular use and treatment approved by the FDA. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of “off-label” uses (*i.e.*, outside the FDA-approved indication, dosing and/or population), and a company that is found to have improperly promoted off-label uses may be subject to significant liability, such as heavy fines, obligation to submit all future promotional material to the FDA’s review before distribution, and other reporting obligations. In addition, any claims a company makes for its products in advertising or promotion must be appropriately balanced with important safety information and otherwise adequately substantiated. Failure to comply with these requirements can result in adverse publicity, warning letters, corrective advertising, injunctions and potential civil and criminal penalties. Government regulators recently have increased their scrutiny of the promotion and marketing of approved products.

In addition, certain products (or classes of products, such as immunosuppressants) that have special problems (particularly ones that may lead to death or serious injury) are required to include warning information displayed within a box in the prescribing information (a so-called “boxed” or “black box warning”). Some products are also subject to certain promotion and advertising restrictions (*e.g.*, they may be the subject of so-called “reminder advertisements”, which are ads that call attention to the name of the product but do not give the product’s use).

### **c. Withdrawal of approval**

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery

of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information, imposition of post-market studies or clinical trials to assess new safety risks, or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-authorization clinical trials;
- refusal of the FDA to approve pending NDAs or BLAs or supplements to approved NDAs or BLAs, or suspension or revocation of product license approvals;
- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

## **11. Federal and state fraud and abuse and data privacy and security laws and regulations**

In addition to FDA restrictions on marketing of pharmaceutical products, federal and state healthcare laws restrict certain business practices in the biopharmaceutical industry. These laws include, but are not limited to, anti-kickback, false claims, data privacy and security, and transparency statutes and regulations.

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration, directly or indirectly, to induce, or in return for, purchasing, leasing, ordering or arranging for the purchase, lease or order of any good, facility, item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term “remuneration” has been broadly interpreted to include anything of value, including for example, gifts, discounts, the furnishing of supplies or equipment, credit arrangements, payments of cash, waivers of payment, ownership interests and providing anything at less than its fair market value. The Anti-Kickback Statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution, the exceptions and safe harbors are drawn narrowly, and the Group’s practices may not in all cases meet all of the criteria for a statutory exception or safe harbor protection. Practices that involve remuneration that may be alleged to be intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Several courts have interpreted the statute’s intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the statute has been violated. The PPACA amended the intent requirement under the Anti-Kickback Statute and criminal healthcare fraud statutes (discussed below) such that a person or entity no longer needs to have actual knowledge of the statute or the specific intent to violate it in order to have committed a violation. In addition, the PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the civil False Claims Act (discussed below). Further, the civil monetary penalties statute imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal False Claims Act prohibits, among other things, any person or entity from knowingly presenting, or causing to be presented, a false or fraudulent claim for payment or approval to the federal government or knowingly making, using or causing to be made or used a false record or statement material to a false or fraudulent claim to the federal government. A claim includes “any request or demand” for money or property presented to the U.S. government. Several pharmaceutical and other healthcare companies have been prosecuted under these laws for, among other things, allegedly providing free product to customers with the

expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of the product for unapproved, and thus non-covered, uses. In addition, the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, and their respective implementing regulations (collectively, "HIPAA"), created federal criminal laws that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third-party payers and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of, or payment for, healthcare benefits, items or services.

Many states have similar fraud and abuse statutes or regulations, including, without limitation, laws analogous to the federal Anti-Kickback Statute and the federal False Claims Act, that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payer. Some of these state laws apply to a broader range of conduct and may not have the same exceptions as analogous federal laws.

The federal Physician Payments Sunshine Act, enacted as part of the PPACA, requires applicable manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to track and annually report to the Centers for Medicare and Medicaid Services ("CMS") payments and other transfers of value provided to physicians and teaching hospitals and certain ownership and investment interests held by physicians or their immediate family members.

In addition, HIPAA imposes specified requirements relating to the privacy, security and transmission of certain individually identifiable health information. HIPAA applies to certain covered entity health care providers, health plans and health care clearinghouses as well as their business associates, which are entities that create, receive, maintain or transmit protected health information in connection with providing a service to or performing an activity for or on behalf of a covered entity. Violations of HIPAA may result in civil and/or criminal penalties and state attorneys general have authority to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts. Further, pharmaceutical companies may also be subject to federal and state laws that govern the privacy and security of other personal information, including federal and state consumer protection laws, state data security laws, and data breach notification laws.

## **12. Healthcare reform in the United States**

In the United States there have been, and the Group expects there will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system. Among policy makers and payers in the United States, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. In March 2010, the President of the United States signed into law the PPACA, which substantially changes the way healthcare will be financed by both governmental and private insurers, and significantly impacts the pharmaceutical industry. Among the provisions of the PPACA of greatest importance to the pharmaceutical industry are the following:

- an annual, nondeductible fee on any entity that manufactures or imports branded prescription drugs and biologic agents, apportioned among these entities according to their sales of branded prescription drugs under certain government healthcare programs, such as Medicare and Medicaid;
- increases in the statutory minimum rebates a manufacturer must pay as a condition to having covered drugs available for payment under the Medicare Part B and Medicaid programs to 23.1% and 13% of the average manufacturer price for branded and generic drugs, respectively;

- expansion of healthcare fraud and abuse laws, including the federal False Claims Act and the federal Anti-Kickback Statute, and the addition of new government investigative powers and enhanced penalties for non-compliance;
- extension of a manufacturer's Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- a new Medicare Part D coverage gap discount program, under which a participating manufacturer must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's out-patient drugs to be covered under Medicare Part D;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new eligibility categories for certain individuals with income at or below 133% of the federal poverty level beginning in 2014;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program, known as the 340B drug pricing program;
- the new requirements under the federal Open Payments program created as part of the Physician Payments Sunshine Act under Section 6002 of the PPACA, which requires manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the U.S. Department of Health and Human Services information related to "payments or other transfers of value" made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals. Applicable manufacturers and applicable group purchasing organizations must also report annually to the U.S. Department of Health and Human Services ownership and investment interests held by physicians (as defined above) and their immediate family members. Data collection for these reporting requirements began on August 1, 2013, and manufacturers were required to submit reports to the U.S. Department of Health and Human Services by March 31, 2014. Since 2015, manufacturers have been required to submit data reports by the 90th day of each calendar year. The U.S. Department of Health and Human Services discloses the information on a public website;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

The PPACA also establishes an Independent Payment Advisory Board ("**IPAB**") to reduce the per capita rate of growth in Medicare spending. IPAB is mandated to propose recommendations to reduce the rate of Medicare spending growth if it is determined that the rate of growth of Medicare expenditures exceeds target growth rates. The IPAB has broad discretion to propose policies to reduce expenditures, which may have a negative impact on payment rates for medical products and services. The threshold for triggering IPAB proposals was not reached in 2016, so no adjustments will be made under the IPAB until 2019 at the earliest. If no IPAB members are nominated, the duties of the IPAB will default to the Secretary of the Department of Health and Human Services.

Additionally, efforts by government officials or legislators to implement measures to regulate prices or payment for pharmaceutical products could adversely affect our business if implemented. There has recently been considerable public and government scrutiny of pharmaceutical pricing and proposals to address the perceived high cost of pharmaceuticals. Adoption of other new legislation at the federal or state level could further affect demand for, or pricing of, our products. We face uncertainties as a result of federal legislative and administrative efforts to repeal, substantially modify or invalidate some or all of the provisions of the PPACA. There can be no assurance that repeal or replacement of the PPACA, if it occurs, will not adversely affect our business and financial results, and we cannot predict how future federal or state legislative or administrative changes relating to healthcare reform will affect our business.

## **B. Authorization procedures in the European Union**

### **1. Clinical trial approval**

Clinical trials performed in member states of the European Union (each, a “**Member State**”) are subject to certain common rules and regulations. At present, clinical trials performed in the European Union are subject to three directives: (i) the Directive 2001/20/EC of the European Parliament and of the Council of 4 April 2001 on the approximation of the laws, regulations and administrative provisions of the Member States relating to the implementation of good clinical practice in the conduct of clinical trials on medicinal products for human use (the “**Clinical Trials Directive**”), (ii) Commission Directive 2003/94/EC of 8 October 2003, laying down the principles and guidelines of good manufacturing practice in respect of medicinal products for human use and investigational medicinal products for human use (the “**GMP Directive**”), and (iii) Commission Directive 2005/28/EC of 8 April 2005 laying down principles and detailed guidelines for good clinical practice (“**GCP**”) as regards investigational medicinal products for human use, as well as the requirements for authorization of the manufacturing or importation of such products (the “**GCP Directive**”). Among others, these directives ensure uniform application of the legislation on clinical trials in the European Union by laying down guidelines regarding applications for and the conduct of clinical trials, the transparency of clinical trials, safety reporting obligations and set up EudraCT, the database of clinical trials in the European Union.

The European Medicines Agency (“**EMA**”) plays a role regarding the uniform application of the above-described directives, although it has no role at all in the authorization of clinical trials, which remains at Member State level. The EMA has a central role in harmonizing and coordinating GCP standards within different Member States, maintains, develops and coordinates the above-mentioned EudraCT database and manages the European Union Clinical Trials Register that provides publicly available information on certain clinical trials.

Pursuant to the Clinical Trials Directive, a system for the approval of clinical trials in the European Union has been implemented through national legislation of the Member States. Under this system, sponsors must seek approval from the competent national authority of any Member State in which a study is planned to be conducted. To this end, a Clinical Trial Application is submitted, which must be supported by an investigational medicinal product dossier and further supporting information prescribed by the Clinical Trials Directive and other applicable guidance documents. Furthermore, a clinical trial may only be started after a competent ethics committee has issued a favorable opinion on the clinical trial application in that country.

A new EU regulation, Regulation EU No. 536/2014 of the European Parliament and of the Council of 16 April 2014 on clinical trials on medicinal products for human use, and repealing Directive 2001/20/EC will provide a more streamlined process for clinical trials in the European Union. At present, the new Regulation entered into force in 2014, but will likely not be applicable until the second half of 2019 or later. After the date of application, a transition period of three years will apply, during which clinical trials in the European Union may continue to be governed by the three directives described above. In particular, clinical trial sponsors will be able to submit new clinical trial applications under either the Directive or the Regulation during the first year of application of the Regulation. During the second and third year of application of the Regulation, new clinical trial applications will have to be submitted under the Regulation, whereas existing clinical trials which application was submitted under the Directive will continue to be regulated under the Directive.

The new Regulation will create a straightforward application process for clinical trial authorizations, their assessment and the public’s access to clinical trial data through one single channel, the new EU Clinical Trial Portal and Database. The assessment of such applications will also be harmonized among Member States to some extent. In particular, for clinical trials to be conducted in multiple EU member states, Part I of the assessment, covering mainly the administrative and scientific aspects, will be carried out by a Reporting member state. Part II of the assessment, covering mainly ethical and national aspects, will be carried out by each member state Concerned individually. Each member state Concerned will then issue a single decision concerning the clinical trial, applicable with respect to its territory. This is expected to provide for

more transparency of the entire process and more legal certainty to the sponsors concerned, although the actual extent of the changes brought by the Regulation remain to be determined in practice. Among other things, it is unclear whether the Regulation will shorten the time frames for approval of new clinical trials in the EU, and whether it will bring more harmonization in the assessment of clinical trial applications in the EU. The Regulation will also streamline sponsors' reporting and notification obligations, which may increase and be more detailed, but will have to be done through the EU Portal, thereby likely facilitating compliance by sponsors with such obligations. Finally, the new Regulation will increase the mandatory public disclosure of data relating to clinical trials, including clinical trial results, given that all data submitted through the EU Portal will be made publicly available through the EU Database, subject to limited exceptions concerning mainly the protection of personal data and commercially confidential information..

## 2. Marketing authorization

Authorization to market a medicinal product in the European Union proceeds under one of four procedures: a centralized authorization procedure, a mutual recognition procedure, a decentralized procedure or a national procedure:

Centralized authorization procedure. The centralized authorization procedure provides for the grant of a single marketing authorization that is valid for all 28 Member States, plus by extension the European Economic Area (the "EEA") member states, Norway, Iceland and Liechtenstein. This procedure results in a single marketing authorization issued by the European Commission (the "EC") that is valid across the EEA. The centralized procedure is mandatory for human medicines that (i) contain a new active substance indicated for the treatment of certain diseases, such as cancer, HIV/AIDS, diabetes, neurodegenerative disorders or autoimmune diseases and other immune dysfunctions, (ii) are derived from biotechnology processes, such as genetic engineering, and (iii) are officially designated orphan medicines. The centralized procedure is optional for those products that are highly innovative or for which a centralized process is in the interest of patients.

Other authorization procedures. In general, if the centralized procedure is not followed, there are three alternative routes to authorize medicinal products in the European Union:

*Decentralized procedure.* Using the decentralized procedure, an applicant may apply for simultaneous authorization in more than one European Union country of medicinal products that have not yet been authorized in any European Union country and that do not fall within the mandatory scope of the centralized procedure. The competent authority of the reference Member State will lead in the assessment of the application.

*Mutual recognition procedure.* In the mutual recognition procedure, a medicine is first authorized in one Member State, in accordance with the national procedures of that country. Following this, further marketing authorizations can be sought from other European Union countries in a procedure whereby the countries concerned agree to recognize the validity of the original, national marketing authorization.

*National procedure.* Applicants following the national procedure will be granted a marketing authorization that is valid only in a single Member State. This procedure is not available for applicants seeking approval in more than one Member State.

In some cases, a Pediatric Investigation Plan (a "PIP"), and/or a request for waiver or deferral, is required for submission prior to submitting a marketing authorization application. A PIP describes, among other things, proposed pediatric trials and their timing relative to clinical trials in adults.

In the European Union, approved drugs are subject to continuing regulation by the regulatory authorities. Consequences of non-compliance with EU post-market obligations are largely similar to those imposed by U.S. regulatory authorities.

### 3. Regulatory data protection

In the European Union, some marketing authorizations benefit from an “8+2(+1)” period of regulatory data protection. This regime consists of a regulatory data protection period of eight years plus a concurrent market exclusivity of ten years. The overall ten-year period will be extended to a maximum of 11 years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications that, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. This data exclusivity prevents a third party from referencing the innovator’s data for eight years, after which generic manufacturers may submit marketing authorization applications referencing the innovator’s data, but the third party cannot market a generic version until the ten- (or 11-)year period has elapsed.

Depending upon the timing and duration of the EU marketing authorization process, products may be eligible for up to five years’ supplementary protection certification (an “SPC”), pursuant to Regulation (EC) No. 469/2009. Such SPCs extend the rights under the basic patent for the drug.

### 4. EMA Orphan designation and exclusivity

In the European Union, Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, complemented by various other regulations, lay down the rules of orphan drug designation. The EMA’s Committee for Orphan Medicinal Products (“COMP”) grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions that affect not more than five in 10,000 persons in the European Union, or when, without incentives, it is unlikely that sales of such products in the European Union may be sufficient to justify the necessary investment in developing the products. Orphan drug designation is only available where no satisfactory method of diagnosis, prevention or treatment of the condition has been authorized (or the product would be a significant benefit to those affected).

In the European Union, orphan drug designation, if maintained by the time of the EC decision on the marketing authorization, entitles a company to financial incentives such as reduction of fees or fee waivers and ten years of market exclusivity following grant of the medicinal product approval. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Two years additional orphan exclusivity protection can be applied for when an applicant has complied with all requirements as set forth in an approved PIP. Market exclusivity would not prevent the approval of a similar drug that is shown to be safer, more effective or otherwise clinically superior.

Companies that classify as small or medium-sized enterprises (“SME”) benefit from further incentives, including administrative and procedural assistance from the EMA’s SME office and fee reductions.

Orphan drug designation must be requested before submitting an application for marketing approval. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. Sponsors must submit orphan drug applications to the EMA. The evaluation process by the COMP takes a maximum of 90 days from validation of the application. The EC will issue a decision on a COMP opinion within 30 days of receipt of such opinion.

A sponsor may file a common application for orphan drug designation in the European Union and in the United States if it wishes to receive orphan drug designation in both territories. In that case, a common application must be filed with both the EMA and the OOPD.

## **5. Exceptional circumstances/conditional approval**

Orphan drugs or drugs with unmet medical needs may be eligible for EU approval under exceptional circumstances or with conditional approval. Approval under exceptional circumstances is used when an applicant is unable to provide comprehensive data on the efficacy and safety under normal conditions of use because the indication for which the product is intended is encountered so rarely that the applicant cannot reasonably be expected to provide comprehensive evidence, when the present state of scientific knowledge does not allow comprehensive information to be provided, or when it is medically unethical to collect such information. An approval under exceptional circumstances must be subject to post-authorization controls or conditions, such as an obligation to conduct further studies, restrictions on supply, use or prescription or special labeling. An approval under exceptional circumstances is based on the assumption that the company will never be able to generate a complete data package. The authorization is valid for the standard five-year period (during which it is reviewed annually), after which it must usually be renewed only once.

A conditional marketing authorization may be applicable to orphan medicinal products, medicinal products for seriously debilitating or life-threatening diseases or medicinal products to be used in emergency situations in response to recognized public threats. Conditional marketing authorization can be granted on the basis of less complete data than is normally required in order to meet unmet medical needs and in the interest of public health, provided the risk-benefit balance is positive, it is likely that the applicant will be able to provide the comprehensive clinical data, and unmet medical needs will be fulfilled. Conditional marketing authorization is subject to certain specific obligations, usually including the obligation to generate and submit additional clinical data, and must be renewed annually until the obligations have been completed and the authorities have reviewed the new data and confirmed the approvability of the product.

## **6. Accelerated assessment**

Under the centralized procedure in the European Union, the maximum timeframe for the evaluation by the EMA's Committee for Medicinal Products for Human Use ("CHMP") of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP). However, the applicant may request an accelerated assessment procedure in order to meet, in particular, the legitimate expectations of patients and to take account of the increasingly rapid progress of science and therapies, for medicinal products of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. Applicants requesting an accelerated assessment procedure must justify that the medicinal product is expected to be of major public health interest. If the CHMP accepts the request, the maximum timeframe for the evaluation of the marketing authorization application is reduced to 150 days, excluding clock stops.

## **7. Post-authorization requirements**

Requirements on drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are similar to those in the United States. Drug manufacturers are required to register their establishments with the local health authorities and state agencies, and are subject to periodic inspections by these authorities and state agencies for compliance with cGMP requirements in order to gain renewal of their manufacturing license. Changes to the manufacturing process are strictly regulated and often require prior regulatory approval before being implemented. Regulations also require investigation and correction of any deviations from cGMP requirements and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

## **8. Marketing Authorization Variation Procedure**

Once a marketing authorization has been granted by the competent authority, variations to the terms of such marketing authorization are possible and may be necessary. Variations include: (i) administrative changes such as a change of company name and/or address; (ii) changes to the characteristics of a product that can

affect its quality, such as a change to its composition; (iii) changes to the safety, efficacy or pharmacovigilance of the product. Changes are classed as minor (type I) or major (type II). Minor changes are either type IA or IB. Type II variations include, among others, variations related to the addition of a new therapeutic indication or to the modification of an existing indication.

Type I variations are subject to a notification procedure.

Type II variations are subject to a 'prior approval procedure' in accordance with Commission Regulation (EC) No 1234/2008 on marketing authorization variations. The CHMP is responsible for drawing up an opinion concerning the variation of the marketing authorization. The CHMP opinion can be either favorable or not favorable to the granting of the requested variation of the marketing authorization by the European Commission. The European Commission takes the final decision on whether or not to grant a variation of a marketing authorization for a medicinal product that was approved under the centralized procedure.

## **9. Procedure for the re-examination of CHMP opinions**

In case of a negative CHMP opinion on a variation application, the marketing authorization holder may request re-examination of the CHMP opinion within 15 days after receipt of the opinion. Within 60 days the marketing authorization holder must submit details grounds for requesting re-examination.

As a general rule, the CHMP must re-examine its opinion and adopt a new opinion within 60 days following receipt of the grounds for the re-examination request. Within 15 days of its adoption, the CHMP must send the final CHMP opinion to the European Commission, the Member States and the marketing authorization holder. The final CHMP opinion can be either favorable or not favorable to the granting of the requested variation of the marketing authorization by the European Commission.

## **10. Pharmacovigilance**

Pharmacovigilance (“**PV**”) refers to the science and activities relating to the detection, assessment, understanding, and prevention of adverse effects or any other medicine-related problem. Underlying objectives for PV are preventing harm from adverse reactions arising from the use of authorized medicinal products (within or outside the terms of the marketing authorization) and promoting the safe and effective use of medicinal products. Marketing authorization holders are subject to detailed and extensive risk management and PV obligations under Directive 2001/83/EC and must comply with Good Pharmacovigilance Practices (“**GVP**”) set out in the “Guidelines on good pharmacovigilance practices” dated 12 October 2017. PV obligations in particular include detailed obligations regarding reporting. For example, a marketing authorisation holder must record all suspected adverse reactions which are brought to its attention and report such information via the centralised Eudravigilance database, and submit periodic benefit-risk evaluation reports (“**PBRERs**”) (previously called periodic safety update reports) to the EMA regarding the benefits and risks of the medicinal product.

Further requirements relate to the implementation of risk minimisation measures on a per product basis (Risk Management System) as well as a requirement to operate a PV system to monitor the safety of authorised medicinal products and to detect any change to their risk-benefit balance. A marketing authorisation holder must also have permanently and continuously at its disposal an appropriately “qualified person responsible for PV” who resides in the EU.

## **11. Advertising and Compliance**

Directive 2001/83/EC sets out strict rules on the advertising of medicinal products. However, the EU legislation gives members states certain flexibility to ensure adequate and effective monitoring of advertising and the advertising rules are consequently not fully harmonised across the EU.

The concept of advertising is broadly defined and includes any form of door-to-door information, canvassing activity or inducement designed to promote the prescription, supply, sale or consumption of medicinal

product. Advertising must not be misleading and there is a positive obligation for the advertising to encourage the rational use of a medicinal product amongst other matters. All promotional materials and activity must also comply with the official Summary of Product Characteristics (“**SmPC**”), as approved by the competent regulatory authority granting the marketing authorization.

The advertising of unauthorised medicines is prohibited. This includes the advertising of an authorised medicine for uses (i.e. therapeutic indications) outside the scope of its marketing authorization (so called “off label”).

In addition, relationships with healthcare professionals and associations are subject to stringent anti-gift statutes, the scope of which differs across the EU. National “Sunshine Acts” also require marketing authorization holders of medicinal products to annually report/publish transfers of value provided to health care professionals and associations.

## **12. Pharmaceutical compounding**

Pharmaceutical compounding is a practice in which a licensed pharmacist prepares medicines in a pharmacy by combining, mixing, or altering pharmaceutical ingredients. In particular, pharmaceutical compounds are formulations developed by licensed (compounding) pharmacists based on active ingredients that are out of patent. Article 3 of the EU Directive 2001/83 provides that the Directive does not apply to:

- any medicinal product prepared in a pharmacy in accordance with a medical prescription for an individual patient (“magistral formula”); or
- any medicinal product which is prepared in a pharmacy in accordance with the prescriptions of a pharmacopoeia and is intended to be supplied directly to the patients served by the pharmacy in question (“official formula”).

In pharmaceutical compounding, the medication is most often tailor-made per patient, and may or may not be specifically prescribed by a physician or other prescriber. The formulation can contain alternative dosages or strengths or can consist of combination therapies not commercially available. Because of the tailor-made character, compounded formulations fall under different regulatory rules than those governing standard drugs produced by pharmaceutical companies.

One of the main reasons for prescribing compounded medications is “patient non-compliance”. This means that a patient is unable to take certain medications in their existing formulations or forms because the patient is allergic to certain preservatives, dyes or other non-essential ingredients, has difficulty taking the standard available forms (such as pills or capsules), or the medication strength is not suitable (for example, for infants or the elderly) or the complexity of the patient’s chronic conditions require customised ready-to-use drug formulations not commercially available.

Compounding pharmacists are able to alter the formulations and/or forms of existing medications, including:

- lowering the level of or eliminating the preservatives, dyes or other ingredient causing allergies;
- increasing or decreasing dosage strengths;
- creating combination therapies, such as for HIV and cancer patients;
- combining multiple pharmaceuticals into a single medication to simplify a dosing regime;
- alternative forms, such as troches, lozenges, candies, gels and liquids, to create a specialised delivery mechanism; and
- added flavours for better taste and easier ingestion.

Pharmaceutical compounding also offers a solution to patients who require medications that have been discontinued by drug manufacturers or can become an alternative for patients who may be facing a supply shortage of their normal commercially available medications.

Pharmaceutical compounds may be competing with Raxone® and may potentially reduce our market share for idebenone.

Some countries (e.g., Belgium and the Netherlands) allow the outsourcing of pharmaceutical compounding by licensed pharmacists to authorized commercial manufacturers, thus enhancing the potential for compounding on a large scale. In addition, given the high prices of certain medicinal products (e.g., especially in oncology or with respect to orphan medicines), certain governments are considering to adopt measures to enlarge the possibility of compounding by pharmacists (e.g., the Netherlands by including an exception in its patent act). Whether compounded medicines can be reimbursed depends on the country.

For a description of the risks that pharmaceutical compounding presents to our business see risk factor “*Pharmacies have been compounding idebenone. Future compounding may adversely affect our sales of Raxone®.*”

### **C. Clinical trials and marketing authorization of medicinal products in other countries**

Although the above discussion focuses on regulation in the United States and the European Union, the Group has filed an MAA for its product candidate Raxone® in DMD in Switzerland and may seek marketing authorizations for its product candidates in other countries. In order to market any product outside of the United States and the European Union, the Group would need to comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of its products. Whether or not the Group obtains FDA or EMA approval for a product, it would need to obtain the necessary approvals by the comparable regulatory authority before it can commence clinical trials or marketing of the product in such other country or jurisdiction outside of the United States and the European Union. Marketing approval in one country or jurisdiction does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country or jurisdiction may negatively impact the regulatory process in others. Generally, the Group expects that its product and product candidates will be subject to regulation in other countries that is similar in nature and scope as those imposed in the United States and the European Union, although there can be important differences.

### **D. Pharmaceutical coverage, pricing and reimbursement**

In the United States, the European Union and other countries and jurisdictions, sales of the Group's product or any product candidates for which the Group may receive marketing approval will depend in part on the availability of coverage and adequate reimbursement to healthcare providers and patients from third-party payers. Third-party payers include government authorities (including government health programs, such as Medicare and Medicaid in the United States), managed care providers, private health insurers and other organizations. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payers to reimburse all or part of the associated healthcare costs. Patients are unlikely to use pharmaceutical products unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of those products. Third-party payers are increasingly focused on containing healthcare costs by challenging the price and examining the cost-effectiveness of medical products and services.

In addition, significant uncertainty exists as to the coverage and reimbursement status of newly approved healthcare product candidates. The market for the Group's product or any product candidates for which the Group may receive marketing approval in the future will depend significantly on the degree to which these products are listed on third-party payers' drug formularies, or lists of medications for which third-party payers provide coverage and reimbursement to the extent products for which the Group may receive marketing approval are covered under a pharmacy benefit or are otherwise subject to a formulary. The industry competition to be included on such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payers may refuse to include a particular branded drug on their formularies or otherwise restrict patient access to a branded drug when a less costly generic equivalent or other alternative is available. Furthermore, third-party payer reimbursement to providers for the Group's product

or any product candidates for which the Group may receive marketing approval may be subject to a bundled payment that also includes the procedure administering such products. To the extent there is no separate payment for any such products, there may be further uncertainty as to the adequacy of reimbursement amounts. In addition, because each third-party payer individually approves coverage and reimbursement levels, obtaining coverage and adequate reimbursement is a time-consuming, costly and sometimes unpredictable process. The Group may be required to provide scientific and clinical support for the use of any product to each third-party payer separately with no assurance that coverage and reimbursement approval would be obtained, and the Group may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost-effectiveness of the Group's products. The Group cannot be certain that its product or any product candidates for which it may receive marketing approval will be considered cost-effective. Further, because coverage and reimbursement determinations are made on a payer-by-payer basis, obtaining acceptable coverage and reimbursement from one payer does not guarantee that the Group will obtain similar acceptable coverage or reimbursement from another payer. This process could delay the market acceptance of the Group's product or any product candidate for which the Group may receive marketing approval and could have a negative effect on the Group's future revenues and operating results. If the Group is unable to obtain coverage of, and adequate reimbursement and payment levels for, its product or any products for which it may receive marketing approval from third-party payers, physicians may limit how much or under what circumstances they will prescribe or administer them and patients may decline to purchase them. This in turn could affect the Group's ability to successfully commercialize its products and impact its profitability, results of operations, financial condition and future success.

Furthermore, in many countries, particularly the countries in the European Union, the pricing of prescription drugs is subject to government control. In some non-U.S. jurisdictions, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, European Union Member States typically have options to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A Member State may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. The Group may face competition for its product or any product candidates for which it may receive marketing approval from lower-priced products in any country that has placed price controls on pharmaceutical products. In addition, there may be importation of foreign products that compete with the Group's product or any products for which it may receive marketing approval, which could negatively impact the Group's profitability.

## **XII. THE ISSUANCE OF THE NEW SHARES**

### **A. In-licensing of ReveraGen's Vamorolone**

As discussed under “—*License and collaboration agreements*” in “*The Company and its Business*” beginning on page 84, on November 20, 2018 we entered into an agreement (the “**Option Agreement**”) with Idorsia Pharmaceuticals Ltd, Allschwil, Switzerland (“**Idorsia**”), under which we have acquired an option from Idorsia for the exclusive sub-license to commercialize ReveraGen’s vamorolone. As compensation for this option, we issued the 1,000,000 Idorsia Shares to Idorsia on November 21, 2018 and have agreed to pay USD 20.0 million in cash. We intend to pay the cash component of the consideration from the proceeds of the sale of the Offered Shares.

### **B. Sourcing of the Idorsia Shares**

On November 21, 2018, the Company issued the Idorsia Shares out of its authorized share capital pursuant to article 3a of its articles of association (the “**Articles**”) as in effect at the time of the subscription. As authorized by the Articles, the Company’s board of directors had excluded the preemptive rights of existing shareholders with respect to the issuance of the Idorsia Shares and allocated them to Idorsia as part of the consideration for the exercise of the option relating to the in-licensing of the Compound under the Sub-license Agreement.

### **C. Sourcing of the Offered Shares**

On December 11, 2018, the shareholders of the Company resolved on an ordinary capital increase of up to CHF 5,000,000 by issuing a maximum of 5,000,000 new shares in the Company with a par value of CHF 1 each (the Offered Shares). The Company intends to use the net proceeds of this Offering to fund the USD 20.0 million payable to Idorsia as the cash component of the consideration for the acquisition of the option for the exclusive sub-license to commercialize ReveraGen’s vamorolone (see “—*License and collaboration agreements*” in “*The Company and its Business*” beginning on page 84 for more information) and any net proceeds from the Offering in excess of that amount for general corporate purposes.

### **D. Lock-ups**

Idorsia has agreed with the Company that, without the prior written consent of the Company and subject to exceptions, it will not sell any Idorsia Shares in the public market or effect certain other transactions in the Idorsia Shares or related to the Idorsia Shares, until the Company obtains FDA approval for sale of vamorolone in DMD in the U.S.

Idorsia will not be subject to any lock-up undertaking with respect to Shares that it may hold or acquire that are not Idorsia Shares.

Further, the Company has agreed with the Managers to a 90-day lock-up commencing on the date of the Accelerated Book-Build Agreement with respect to certain transactions in Shares and share-based instruments. See “—*Lock-up arrangements*” in “*Offering and Sale*” beginning on page 147.

### **XIII. RELATED PARTY TRANSACTIONS**

For information on the Option Agreement with Idorsia, see “*The Company and its Business—License and collaboration agreements*” beginning on page 84 and “*The Issuance of the New Shares*” beginning on page 117.

For information on the compensation and participations of, and other transactions with, the members of the Board and the Executive Management see the respective subsections of “*Board of Directors and Executive Management*” beginning on page 122.

## XIV. PRINCIPAL SHAREHOLDERS

The following table and the footnotes thereto disclose significant shareholders and significant groups of shareholders of the Company and their shareholdings (including purchase and sale positions) according to the Federal Act on Financial Market Infrastructures and Market Conduct in Securities and Derivatives Trading of June 19, 2015 (the “FMIA”) and its implementing ordinance, to the extent that the Company is aware of them. Other than with respect to the Company, the members of the board of directors and of executive management, this information is provided based on the notifications received by the Company in accordance with article 120 et seq. of the FMIA from the relevant shareholders and groups of shareholders. The shareholdings (including purchase and sale positions) of such shareholders may have changed since the date of their respective notification.

Each of the two following tables take into consideration the registration of the Idorsia Shares in the commercial register.

Each Share carries one vote at the general meeting of shareholders of the Company. Therefore, the number of Shares held by each shareholder of the Company set forth below is equal to the number of voting rights held by such shareholder.

### A. As of the date of this Offering Memorandum

The percentages set forth in the table below are calculated on the basis of the 7,527,479 Shares recorded in the commercial register immediately prior to the registration of the Offered Shares.

Direct shareholder	Shares	% of voting rights (non-diluted)	Delegated voting rights	% of voting rights (non-diluted)	Other purchase positions	% of voting rights (non-diluted)	Aggregate % of voting rights (non-diluted)	Sale positions	% of voting rights (non-diluted)
Idorsia Pharmaceutical AG, Allschwil, Switzerland <sup>(1)</sup>	1,000,000	13.28					13.28		
Iglu Group AG, Lucerne, Switzerland <sup>(2)</sup>	557,350	7.40					7.40		
Waypoint Growth LP, St Helier, Jersey, Channel Islands <sup>(3)</sup>	545,777	7.25					7.25		
RTW Master Fund, Ltd., Georgetown, Grand Cayman, Cayman Islands; RTW Innovation Master Fund, Ltd., New York, USA <sup>(4)</sup>	325,638	4.33					4.33		
Members of the Board and of Executive Management <sup>(5)</sup>	117,261	1.56			1,008,301	13.39	14.95		
Company <sup>(6)</sup>	57,511	0.76					0.76	1,934,221	25.70

(1) As per the disclosure notice published on November 29, 2018.

(2) As per the disclosure notice published on March 2, 2018. The beneficial owners of the respective positions are Ralf Arnold, Lucerne, Switzerland; Markus Kühnle, Efringen-Kirchen, Germany; and Thomas Terhorst, Lucerne, Switzerland. The representative of the group is Ralf Arnold, Lucerne, Switzerland.

(3) As per the disclosure notice published on February 27, 2018. The beneficial owners of the respective positions are Ernesto Bertarelli and Donata Guichard-Bertarelli, both Gstaad, Switzerland. The beneficial owners are bound by a shareholders’ agreement. The representative of the group is Bemido SA, Geneva, Switzerland.

(4) As per the disclosure notice published on October 30, 2018. The beneficial owner of the respective positions is Roderick Wong, New York, USA.

(5) The members of the Board and of the Executive Management do not act in concert within the meaning of article 121 FMIA. None of the members of the Board or of the Executive Management holds at least 3% of the voting rights of the Company. The purchase positions of these individuals comprise (a) an aggregate of 266,587 stock-settled American-type call options with a subscription ratio of 1:1 written by the Company in connection with its stock option plans (see “—Stock Option Plans (BSOPs and ESOPs)” in “The Company and its Business” beginning on page 93 for more information on such plans and “—Options, warrants and conversion rights” in “Description of the Company’s Capital Structure and Shares” beginning on page 136 for details on the

- outstanding stock options); and (b) an aggregate of 741,714 stock-settled share appreciation rights written by the Company in connection with its share appreciation rights plans (see “—Share Appreciation Rights Plans (BSARPs and ESARPs)” beginning on page 93 for more information on such plans and “—Options, warrants and conversion rights” in “Description of the Company’s Capital Structure and Shares” beginning on page 136 for details on the outstanding rights).
- (6) The Company’s sale positions comprise (a) an aggregate of 266,587 stock-settled American-type call options with a subscription ratio of 1:1 written by the Company in connection with its stock option plans (see “—Market exclusivity and intellectual property” beginning on page 90 for more information on such plans and “—Options, warrants and conversion rights” beginning on page 136 for details on the outstanding stock options); (b) an aggregate of 741,714 stock-settled share appreciation rights written by the Company in connection with its share appreciation rights plans (see “—Market exclusivity and intellectual property” beginning on page 90 for more information on such plans and “—Options, warrants and conversion rights” beginning on page 136 for details on the outstanding rights); and (c) conversion rights for up to 925,920 Shares attached to the 12,000 Bonds (see “—CHF 60 million Senior Unsecured Convertible Bonds 2017-2022” in “The Company and its Business” beginning on page 88 for more information).

## B. Upon completion of the Offering

The following table describes the shareholdings as of the first trading day of the Offered Shares. The figures set forth in the table below are calculated based on the assumption that all Offered Shares will be sold in the Offering and that none of the shareholders listed below will purchase any Shares in the Offering. The percentages set forth in the table below are calculated on the basis of the maximum number of 12,527,479 Shares expected to be recorded in the commercial register as of the first trading day of the Offered Shares, *i.e.*, including the maximum number of 5,000,000 Offered Shares approved by the extraordinary shareholders’ meeting held on December 11, 2018.

Direct shareholder	Shares	% of voting rights (non-diluted)	Delegated voting rights	% of voting rights (non-diluted)	Other purchase positions	% of voting rights (non-diluted)	Aggregate % of voting rights (non-diluted)	Sale positions	% of voting rights (non-diluted)
Idorsia Pharmaceutical AG, Allschwil, Switzerland <sup>(1)</sup>	1,000,000	7.98					7.98		
Iglu Group AG, Lucerne, Switzerland <sup>(2)</sup>	557,350	4.45					4.45		
Waypoint Growth LP, St Helier, Jersey, Channel Islands <sup>(3)</sup>	545,777	4.36					4.36		
RTW Master Fund, Ltd., Georgetown, Grand Cayman, Cayman Islands; RTW Innovation Master Fund, Ltd., New York, USA <sup>(4)</sup>	325,638	2.60					2.60		
Members of the Board and of Executive Management <sup>(5)</sup>	117,261	0.94			1,008,301	8.05	8.98		
Company <sup>(6)</sup>	57,511	0.46					0.46	1,934,221	15.44

(1) As per the disclosure notice published on November 29, 2018.

(2) As per the disclosure notice published on March 2, 2018. The beneficial owners of the respective positions are Ralf Arnold, Lucerne, Switzerland; Markus Kühnle, Efringen-Kirchen, Germany; and Thomas Terhorst, Lucerne, Switzerland. The representative of the group is Ralf Arnold, Lucerne, Switzerland.

(3) As per the disclosure notice published on February 27, 2018. The beneficial owners of the respective positions are Ernesto Bertarelli and Donata Guichard-Bertarelli, both Gstaad, Switzerland. The beneficial owners are bound by a shareholders’ agreement. The representative of the group is Bemido SA, Geneva, Switzerland.

(4) As per the disclosure notice published on October 30, 2018. The beneficial owner of the respective positions is Roderick Wong, New York, USA.

(5) The members of the Board and of the Executive Management do not act in concert within the meaning of article 121 FMIA. None of the members of the Board or of the Executive Management holds at least 3% of the voting rights of the Company. The purchase positions of these individuals comprise (a) an aggregate of 266,587 stock-settled American-type call options with a subscription ratio of 1:1 written by the Company in connection with its stock option plans (see “—Stock Option Plans (BSOPs and ESOPs)” in “The Company and its Business” beginning on page 93 for more information on such plans and “—Options, warrants and conversion rights” in “Description of the Company’s Capital Structure and Shares” beginning on page 136 for details on the outstanding stock options); and (b) an aggregate of 741,714 stock-settled share appreciation rights written by the Company in connection with its share appreciation rights plans (see “—Share Appreciation Rights Plans (BSARPs and ESARPs)” beginning on page 93 for more information on such plans and “—Options, warrants and conversion rights” in “Description of the Company’s Capital Structure and Shares” beginning on page 136 for details on the outstanding rights).

- (6) The Company's sale positions comprise (a) an aggregate of 266,587 stock-settled American-type call options with a subscription ratio of 1:1 written by the Company in connection with its stock option plans (see "*Market exclusivity and intellectual property*" beginning on page 90 for more information on such plans and "*Options, warrants and conversion rights*" beginning on page 136 for details on the outstanding stock options); (b) an aggregate of 741,714 stock-settled share appreciation rights written by the Company in connection with its share appreciation rights plans (see "*Market exclusivity and intellectual property*" beginning on page 90 for more information on such plans and "*Options, warrants and conversion rights*" beginning on page 136 for details on the outstanding rights); and (c) conversion rights for up to 925,920 Shares attached to the 12,000 Bonds (see "*CHF 60 million Senior Unsecured Convertible Bonds 2017-2022*" in "*The Company and its Business*" beginning on page 88 for more information).

### **C. Lock-up**

The Company has agreed with the Managers to a 90-day lock-up commencing on the date of the Accelerated Book-Build Agreement with respect to certain transactions in Shares and share-based instruments. See "*Lock-up arrangements*" in "*Offering and Sale*" beginning on page 147. The positions of the Company and the corresponding percentages of voting rights as of the date of this Offering Memorandum and upon completion of the Offering are set forth in the tables in the two previous sections.

Further, Idorsia has agreed with the Company that, without the prior written consent of the Company and subject to exceptions, it will not, until the Company obtains FDA approval for the sale of vamorolone in DMD, sell any Idorsia Shares in the public market or effect certain other transactions in the Idorsia Shares or related to the Idorsia Shares (see "*Lock-up*" in "*The Issuance of the New Shares*" beginning on page 117 for more details)

### **D. Disclosure of the Managers**

See "*Placement of Offered Shares*" in "*Offering and Sale*" beginning on page 147 for the disclosure of the Managers and the Company in relation to the placement of the Offered Shares and related transactions.

## XV. BOARD OF DIRECTORS AND EXECUTIVE MANAGEMENT

*Unless otherwise noted, the summary below is based on the versions of the articles of association, organizational regulations and other internal regulations that are in effect as of the date of this Offering Memorandum.*

### A. The Board of directors

#### 1. General information

The Company's articles of association (*Statuten*) (the "**Articles**") provide that the board of directors (*Verwaltungsrat*) of the Company (the "**Board**") shall consist of a maximum of eight members. As of the date of this Offering Memorandum, the Board has five members (each, a "**Director**"). The chairman of the Board (the "**Chairman**") and the other Directors are appointed and removed exclusively by the general meeting of shareholders. The maximum term of office for a Director is the time period starting at his election and ending upon completion of the following annual general meeting of shareholders (*ordentliche Generalversammlung*, "**AGM**"). Re-election is permitted.

The Board is responsible for the ultimate direction of the Company's business and the supervision of the persons entrusted with the Company's management. The Board represents the Company vis-à-vis third parties and manages all matters that have not been delegated to another corporate body by law, the Articles, the organizational regulations (*Organisationsreglement*) (the "**Organizational Regulations**") or other regulations. The Board's non-transferable and inalienable duties include:

1. the ultimate direction of the Company and the issuance of the necessary directives in this regard;
2. the determination of the organization of the Company;
3. the administration of accounting, financial control and financial planning;
4. the appointment and removal of the persons entrusted with the executive management and their representation of the Company;
5. the ultimate supervision of the persons entrusted with the management of the Company, in particular with respect to their compliance with laws, the Articles, regulations and directives;
6. the preparation of the annual report, the compensation report and the general meeting of shareholders as well as the implementation of shareholder resolutions;
7. the determination on the payments on the shares that had not previously been fully paid in and on the resulting amendments to the Articles
8. the adoption of resolutions concerning an increase of the share capital to the extent that such power is vested in the Board and of resolutions concerning the consummation of capital increases, the preparation of the capital increase reports and corresponding amendments to the Articles;
9. the examination of the professional qualifications of the auditors;
10. the notification of the court if liabilities exceed assets;
11. the non-delegable and inalienable duties and powers of the Board pursuant to the Federal Act on Merger, Demerger, Transformation and Transfer of Assets (the "**Merger Act**"), any other applicable laws and the Articles.

The Board elects from among its members a vice-chairman (the "**Vice-Chairman**"), and also appoints a secretary (the "**Secretary**"), who needs not be a member of the Board. According to the Articles and the Organizational Regulations, the Board meets at the invitation of the Chairman or, if he is unable to do so, of the Vice-Chairman or the Secretary, as often as required, or whenever a Director indicating the reasons so requests in writing. Board resolutions are passed by a majority of the votes cast. In the case of a tie, the Chairman has the deciding vote. Subject to certain exceptions, the Board is quorate when a majority of its

members are present. Resolutions may be passed by way of circulation in writing, provided that no Director requests oral deliberations or a meeting.

In accordance with and subject to Swiss law, the Articles and the Organizational Regulations, the Board has delegated the Company's management to the the delegate of the Board and chief executive officer of the Company (the "**Delegate of the Board and CEO**"), who leads the top tier of the Company's executive management (the "**Executive Management**"). For more information on the Executive Management see "*The Executive Management*" beginning on page 125.

## 2. Board committees

The Board has established an audit committee (the "**Audit Committee**") and a compensation committee (the "**Compensation Committee**").

### a. Audit Committee

The chairman and the other member(s) of the Audit Committee are appointed by the Board. As per the Company's audit committee charter (the "**Audit Committee Charter**"), the Audit Committee shall comprise at least two members of the Board all of which shall be independent, non-executive members of the Board.

The Audit Committee recommends for approval by the Board the Company's annual and half year interim financial statements. The Audit Committee discusses the following matters with management and the auditors: significant financial reporting issues and judgments made in connection with the preparation of the Company's financial statements, including any significant changes in the Company's selection or application of accounting principles; any major issues as to the adequacy of the Company's internal controls; any special audit steps adopted in light of material control deficiencies; the Company's financial budgets and programs for compliance with the financial disclosure requirements of applicable law; the Company's processes to maintain an adequate system of internal controls; and the Company's risk assessment and risk management policies.

The Audit Committee holds at least four meetings per year and additional meetings as needed or requested by any of its members.

The Audit Committee currently consists of Martin Gertsch (chairman) and Philipp Gutzwiller.

### b. Compensation Committee

The Ordinance against Excessive Compensation in Public Corporations (*Verordnung gegen übermässige Vergütungen bei börsenkotierten Aktiengesellschaften*) of November 20, 2013 (the "**Compensation Ordinance**") requires that the articles of association of a Swiss public company contain provisions regarding, among other things, the duties and responsibilities of its compensation committee.

As required by the Compensation Ordinance, the members of the Compensation Committee are appointed by the Company's general meeting of shareholders for a term of office extending until completion of the following AGM. Re-election is permitted. According to the Articles, the Compensation Committee shall have at least two members. In case of vacancies on the Compensation Committee, the Board shall appoint from among its members substitutes for a term of office extending until completion of the following AGM.

According to the charter of the Compensation Committee, the Compensation Committee is responsible for reviewing the compensation system applicable to the members of the Board and of the Executive Management on an annual basis and ensuring that the Company's regulations and the Articles are compliant with the requirements of applicable laws and regulations as well as Swiss and international corporate governance practices. According to the Articles, the Compensation Committee shall recommend to the Board for approval to propose to the shareholders the total compensation for the Board members (fix cash and equity compensation) and for executive management (fix base salary, a variable cash bonus, equity compensation,

pensions and any other benefits). It shall also propose to the Board candidates for re-election and election by the shareholders and propose executive management candidates for hiring or dismissal. The Board may assign other tasks to the Compensation Committee.

The Compensation Committee holds at least two meetings per year and additional meetings as needed or requested by any of its members.

The Compensation Committee currently consists of Patrick Vink (chairman) and Elmar Schnee.

### 3. Members of the Board

The following table sets forth the name, function and committee membership of each Director on the date of this Offering Memorandum, followed by a short description of each Director's business experience and education.

As of the date of this Offering Memorandum, all Directors other than Thomas Meier are non-executive Directors. Other than disclosed below, none of the Directors has any significant business connections with the Company.

<b>Name</b>	<b>Function</b>	<b>Committee memberships</b>	<b>First elected</b>	<b>End of current term</b>
Elmar Schnee	Chairman of the Board	Member of the Compensation Committee	2017	2019
Martin Gertsch	Vice Chairman of the Board	Chairman of the Audit Committee	2006	2019
Philipp Gutzwiller	Member of the Board	Member of the Audit Committee	2017	2019
Thomas Meier	Delegate of the Board and CEO	–	2017	2019
Patrick Vink	Member of the Board	Chairman of the Compensation Committee	2017	2019

The business address of each Director is Santhera Pharmaceuticals Holding AG, Hohenrainstrasse 24, 4133 Pratteln, Switzerland.

**Elmar Schnee** is Advisor to Management of MindMaze, a neuro-technology company spun off from the Swiss Federal Institute of Technology in Lausanne (EPFL). Prior to that, he was chairman, CEO and board member of Cardiorientis in Zug, Switzerland. Previously, he was a general partner and member of the executive board of Merck KGaA, responsible for its worldwide pharmaceutical business. He also led the major restructuring of the business including the acquisition and integration of Serono. Prior to Merck, Elmar Schnee held senior roles in marketing, licensing, strategy, business development, and as Managing Director with UCB Pharma, Sanofi-Synthelabo, Migliara Kaplan and Fisons. He currently serves on the board of directors of listed Jazz Pharmaceuticals and Stallergenes Greer as well as several privately held life science companies.

**Martin Gertsch** is an experienced board member and financial advisor in the life sciences industry. Up to January 2014, he served as Chief Financial Officer of Acino Holding. Prior to this, he was Vice President Head of Finance EMEA at Synthes and held Chief Financial Officer and Chief Operating Officer positions at Delenex Therapeutics and ESBATech, two privately held biotech companies. From 2002 to the beginning of 2006, he was Chief Financial Officer of Straumann, which he had joined in 1997 as Head of Group Controlling and Reporting. Between 1986 and 1997, Martin Gertsch was an audit engagement manager at PricewaterhouseCoopers, Basel, Switzerland. Martin Gertsch is a Swiss-certified fiduciary and Swiss-certified public accountant. He has also completed several executive-level development programs at IMD (the

International Institute for Management Development) in Lausanne, Switzerland. He serves as a board member of Evolva Holding, and the University Center of Dentistry, Basel (UZB). He is also chairman of the board of two diagnostic start-up companies.

**Philipp Gutzwiller** is Global Head Healthcare at Lloyds Banking Group plc in London. He has accumulated over 15 years of experience as a banker to the broader healthcare industry, advising corporate and private equity clients on the assessment, financing and execution of acquisitions and capital market transactions. He started his career at Roche as a financial controller and later worked as an executive in Roche's corporate mergers and acquisitions team.

**Thomas Meier** was appointed CEO of Santhera in October 2011, having served as its Chief Scientific Officer (CSO). Thomas Meier was the founder and CEO of MyoContract, a Basel-based research company focused on orphan neuromuscular diseases, which he merged with Graffinity Pharmaceuticals of Heidelberg, Germany, in 2004, to form today's Santhera. In 1999, Thomas Meier became an independent research group leader and lecturer in the Department of Pharmacology and Neurobiology at the University of Basel, Switzerland, where he established MyoContract – the first start-up company originating from the Bi-zentrum research facility. Thomas received his PhD in biology from the University of Basel in 1992 and subsequently joined the University of Colorado Health Sciences Center, Denver, CO, USA. He has a distinguished scientific track record in the field of neuromuscular research. Before joining the industry, Thomas was awarded the International Research Fellowship Award from the U.S. National Institutes of Health (NIH) and a long-term fellowship from the Human Frontier Science Program. In 2007, he received the BioValley Basel Award for his outstanding contributions to the life sciences in the area.

**Patrick Vink, MD**, was an advisor to Santhera's Board from 2016 until his appointment to the Board. He has over 25 years of life science industry experience. In his latest assignment, he was employed as Chief Operating Officer at Cubist Pharmaceuticals, overseeing all worldwide commercial and technical operations as well as global alliance management. Previously, Patrick Vink held several senior management positions with Mylan Inc., Novartis Generics/Sandoz, Biogen and Sanofi Synthelabo. He serves as chairman of the listed companies Acacia Pharma, Targovax and Arch Biopartners as well as the privately held company NMD Pharma and is a member of the board of directors of several privately held life science companies.

#### **4. Convictions/proceedings**

There have been no convictions or sanctions against any of the Directors listed in “—*Members of the Board*” beginning on page 124 for finance or business-related crimes in the last five years, and no legal proceedings against any such Director by statutory or regulatory authorities (including designated professional associations) are ongoing.

### **B. The Executive Management**

#### **1. General information**

As per the Organizational Regulations, the Executive Management consists of those functions that have been determined by the Board. The Board appoints and removes members of the Executive Management upon proposal of the Compensation Committee and the CEO.

The Delegate of the Board and CEO chairs the Executive Management and is responsible for, among other things, the achievement of the short and mid-term goals defined by the Board, and is responsible for the proper functioning of Executive Management.

Under the leadership of the Delegate of the Board and CEO, the Executive Management conducts the day-to-day management of the Group in accordance with the Organizational Regulations. The members of the Executive Management report to the Delegate of the Board and CEO.

The Executive Management resolves on its own organization.

## 2. Members of the Executive Management

The following table sets forth the name and principal position of each member of the Executive Management as of the date of this Offering Memorandum, followed by a short description of each member's business experience and education.

<b>Name</b>	<b>Function</b>
Thomas Meier	Delegate of the Board and Chief Executive Officer(1)
Christoph Rentsch	Chief Financial Officer
Oliver Strub	Group General Counsel & Secretary to the Board, Executive Vice President
Günther Metz	Head Business Development, Executive Vice President
Kristina Sjöblom Nygren	Chief Medical Officer & Head Development, Executive Vice President

(1) Mr. Meier's responsibilities include those of a Chief Scientific Officer.

The business address for each member of the Executive Management is Santhera Pharmaceuticals Holding AG, Hohenrainstrasse 24, 4133 Pratteln, Switzerland.

**Thomas Meier**—see “—*Members of the Board*”.

**Christoph Rentsch** joined Santhera as its Chief Financial Officer in 2015. With a background in finance and long-standing experience in the pharmaceutical industry, he brings a profound knowledge of the international public and private funding markets to Santhera. Christoph Rentsch started his career in investment banking at Credit Suisse. Subsequently, he worked in various senior management functions for the Alusuisse-Lonza Group both in Switzerland and in the U.S. As Head of Group Funding and Capital Markets at Roche, he was responsible for all finance transactions on group level for more than 8 years. In 2003, he became partner of Caperis Ltd, an investment advisory and management firm, before joining privately held Polyphor as Chief Financial Officer, where he supported the company in key stages of its development. Christoph Rentsch holds a degree in Economics and Business Administration from the University of Applied Sciences, Basel.

**Oliver Strub** joined Santhera as its General Counsel in 2006 shortly before the Company listed its shares on the SIX Swiss Exchange. Oliver Strub is an experienced commercial lawyer and is responsible for Santhera's general legal affairs, insurance, trademarks, facility management, and IT. From 1995 to 2006, Oliver Strub was with Ciba-Geigy, then Ciba Specialty Chemicals (now part of BASF), where he was Head of Corporate Law and Chief Compliance Officer. He holds a degree in law.

**Günther Metz** was appointed Head of Business Development of Santhera in 2015. He joined Santhera in 2004, the year of its inception, and served as its Vice President Business Development between 2008 and 2015. Günther Metz has spent over 20 years in the life sciences industry and began his career in drug discovery at the French company Fournier Pharma and later joined the German start-up Graffinity Pharmaceuticals. Günther Metz held various research management positions in cross-functional teams, gaining broad experience across the preclinical and clinical pharmaceutical value chain. He received his PhD in biophysics from the University of Freiburg, Germany, in 1992, and subsequently held a postdoctoral research position at Yale University, USA, supported by a fellowship from the Alexander von Humboldt Foundation.

**Kristina Sjöblom Nygren** joined Santhera as Chief Medical Officer (CMO) and Head of Development in January 2017. She studied chemistry and biochemistry and graduated as a medical doctor from the Karolinska Institute, Sweden. She has over 18 years of experience as biopharmaceutical executive in drug development across multiple therapeutic areas, including orphan diseases. During her career, she worked in clinical development roles at Wyeth, AstraZeneca and Biovitrum. Prior to joining Santhera, Kristina served as

VP and Head of Clinical Development at Swedish Orphan Biovitrum AB (Sobi), where she led the clinical development of all programs from first in man to commercialization and life cycle management.

On March 20, 2018, the Company announced that Giovanni Stropoli had stepped down from his role as Chief Commercial Officer Europe & Rest of the World and member of the Executive Management after the Company's decision to reorganize the management of its commercial operations.

### **3. Convictions/proceedings**

There have been no convictions or sanctions against any of member of the Executive Management listed in “—*Members of the Executive Management*” beginning on page 126 for finance or business-related crimes in the last five years, and no legal proceedings against any such member by statutory or regulatory authorities (including designated professional associations) are ongoing.

## **C. Board and Executive Management compensation**

### **1. Legal framework and its implementation in the Articles**

With regard to its compensation arrangements and other corporate governance items, the Company is subject to the Compensation Ordinance and the Directive on Information Relating to the Corporate Governance issued by the SIX Swiss Exchange (the “**Corporate Governance Directive**”).

#### *Say on pay*

The Compensation Ordinance requires a “say on pay” approval mechanism for the compensation of the Board and the Executive Management pursuant to which the shareholders must vote on the compensation of the Board and the Executive Management on an annual basis. In accordance with these requirements, the Articles provide that the general meeting of shareholders must, each year, vote separately on the proposals by the Board regarding the aggregate amounts of:

- (a) the maximum fixed and variable compensation of the Board until the next AGM;
- (b) the maximum fixed compensation of the Executive Management for the following financial year; and
- (c) the maximum variable compensation of the Executive Management for the preceding financial year.

In addition, and on a voluntary basis, the Company submits its compensation report to the AGM for an advisory vote.

If the general meeting of shareholders does not approve a proposal of the Board, the Board determines the (maximum) aggregate amount or several (maximum) partial amounts taking into account all relevant factors and submits such amounts for approval by the same general meeting of shareholders, an extraordinary general meeting of shareholders or the next AGM.

If the aggregate amount of compensation of the Executive Management already approved by the general meeting of shareholders is not sufficient to also cover the compensation of persons newly appointed to or promoted within the Executive Management, each such person may be paid up to 50% of the aggregate amounts of compensation of the Executive Management last approved by the general meeting of shareholders.

#### *Compensation principles in the Articles*

The Compensation Ordinance requires the Company to define in its Articles the principles for the determination of the compensation of the Board and the Executive Management. According to the Articles, the compensation may consist of fix and of short- and long-term variable elements. While the fixed compensation and the short-term variable compensation element are paid in cash (in the form of a base salary and

a cash bonus), the long-term variable element is in the form of equity rights (formerly options, and currently share appreciation rights (“SARs”)).

The variable compensation is paid taking into consideration the achievement of personal and individual goals.

In accordance with the Compensation Ordinance, the Articles provide that the aggregate amount of credits to a particular member of the Board or of the Executive Management must not exceed two times the last annual compensation paid to such individual. Further, the Articles provide that the value of post-retirement benefits beyond occupational pensions must not exceed 100% of the last annual compensation paid to such individual. In the case of lump-sum payments, the value of such post-retirement benefit has to be determined based on recognized actuarial methods.

#### ***Prohibited forms of compensation***

The Compensation Ordinance prohibits certain types of compensation arrangements with members of a Swiss public company’s board of directors and executive management. In particular, the Compensation Ordinance broadly prohibits severance payments in any form. In addition, notice periods in employment agreements exceeding one year and employment agreements for a fixed term of more than one year are deemed to be prohibited severance payments. Post-employment non-compete covenants and consultancy agreements are not subject to the Compensation Ordinance’s severance pay prohibition, unless they are deemed to be disguised severance payments based on their terms. The Compensation Ordinance also restricts certain forms of advance compensation. The decisive element in distinguishing prohibited advance payments from certain types of other advance payments, such as sign-on bonuses, is the point in time at which such payment is made. Consequently, sign-on bonuses compensating benefits and other entitlements that executives forfeit from their previous employers are permissible, whereas genuine prepayments of salary (*i.e.*, if the contractual salary is paid in advance) are not permitted. The Compensation Ordinance also prohibits certain types of transaction bonuses and certain other types of compensation and benefits not expressly provided for by the company’s articles of association.

#### ***Compensation disclosure***

The Compensation Ordinance requires the Board to prepare an annual audited compensation report disclosing the compensation and loans directly or indirectly awarded or granted to members of the Board and the Executive Management (and, to the extent not in line with market standards, to former members of these bodies) during the past financial year. Such compensation and loans also have to be disclosed individually and on a named basis for each member of the Board and for the highest paid member of the Executive Management. In addition, the Company has to disclose the Shares and other equity-linked positions held by members of the Board and of the Executive Management or persons closely related to them in the notes to its annual financial statements.

Furthermore, the Corporate Governance Directive requires the Company to disclose the basic principles and elements of compensation and equity plans for current and former and former members of the Board and of the Executive Management as well as the authority and procedures for determining such compensation.

The Company’s most recent compensation report is included in the annual report for the financial year 2017 (beginning on page 78 thereof) included elsewhere in this Offering Memorandum.

#### ***Criminal provisions***

Members of the board of directors or of the executive management who pay or receive impermissible forms of compensation and thereby act against their “better knowledge” (*wider besseres Wissen*) are liable to imprisonment and a fine. Members of the board of directors who do not comply with certain other provisions of the Compensation Ordinance against their “better knowledge” are liable to imprisonment and/or a fine.

## 2. The Group's compensation policy

The Group's compensation policy for its Board and Executive Management is designed to attract, motivate and retain talent in order to support the achievement of the Company's financial and strategic objectives and also to ensure that the total compensation package is fair and competitive. By combining short- and long-term incentive elements, the Board believes that the compensation system is designed in a way that the interests of the management are aligned with the interests of the Company and its shareholders. Compensation elements are focused on rewarding the delivery of outstanding and sustainable results without inappropriate risk-taking.

The Compensation Committee reviews and monitors the Company's compensation policy in light of the Company's business strategy, corporate goals and values and on an ongoing basis, in order to ensure the alignment of employee interests with those of the shareholders.

## 3. Compensation of non-executive Directors

The compensation for non-executive members of the Board currently consists of annual cash fees and annual grants of SARs (see "*Incentive compensation*" beginning on page 93 for more information on the SARs). Before July 1, 2016, the Company issued stock options instead of SARs (see "*Incentive compensation*" beginning on page 57 for more information). Neither the cash fees nor the SAR allocation depend on the achievement of corporate goals or the individual performance of a non-executive Director. Additionally, the Company pays the employer's social security contributions due on Directors' compensation amounts. Non-executive Directors do not receive any variable compensation.

For the compensation of the members of the Board for the financial years 2017 and 2016 see the Company's compensation report (at page 83 of the annual report for the financial year 2017 included elsewhere in this Offering Memorandum).

For the financial year 2017, the compensation of the members of the Board increased significantly compared to the financial year 2016, primarily as a result of the increase of the number of Directors from two to five (including the Delegate of the Board and CEO) as per the 2017 AGM and the establishment of the Audit Committee. The 2018 AGM has approved an aggregate maximum amount of compensation of the members of the Board of CHF 1,001,000 for the period between the 2018 AGM and the 2019 AGM. The table below shows the maximum compensation of the different functions of the non-executive Directors for such period:

<b>Function</b>	<b>Maximum compensation (CHF)</b>	<b>Number</b>	<b>Total maximum compensation (CHF)</b>
Chairman of the Board	286,000	1	286,000
Vice-Chairman of the Board	242,000	1	242,000
Member of the Board	198,000	2	396,000
Chairman of the Audit Committee	33,000	1	33,000
Member of the Audit Committee	11,000	1	11,000
Chairman of the Compensation Committee	22,000	1	22,000
Member of the Compensation Committee	11,000	1	11,000
<b>Total</b>			<b>1,001,000</b>

Note: The Delegate of the Board and CEO does not receive a separate compensation as a Director.

The Delegate of the Board and CEO (whose compensation is included in the compensation of the Executive Management, see "*Compensation of Executive Management*" beginning on page 130) does not receive a separate compensation as a member of the Board.

The total compensation of the non-executive members of the Board is made 50% in the form of cash (including social security contributions) and 50% in the form of SARs. To calculate the number of SARs to be allocated, the total SAR amount of CHF 500,500 was divided by the fair value of the SARs as of April 13, 2018. The fair value was calculated based on the share price on the trading day at the grant day, then applying the Hull-White model (excluding employers' social security contributions).

Between January 1, 2018, and September 30, 2018, the Company granted the non-executive members of the Board a total of 62,659 SARs with exercise prices between CHF 16.20 and CHF 36.70.

#### **4. Compensation of Executive Management**

The compensation for members of the Executive Management currently consists of fixed compensation and variable compensation.

The fixed compensation includes a base salary, social security contributions and payments to the pension fund by the Company. The base salary takes into account the position, responsibilities, experience and skills of the respective individual. Base salaries are reviewed annually by the Compensation Committee, taking into account individual performance and the results of the external benchmarking.

The variable compensation comprises an annual cash bonus as short-term incentive and annual SAR grants as long-term incentive. Before July 1, 2016, the Company issued stock options instead of SARs. See “—*Incentive compensation*” in “*The Company and its Business*” beginning on page 57 for more information. For the compensation of the members of the Executive Management for the financial years 2017 and 2016 see the Company's compensation report (at page 87 of the annual report for the financial year 2017 included elsewhere in this Offering Memorandum).

For the fixed compensation for the financial year 2018, the 2017 AGM has approved a maximum aggregate amount of CHF 3,200,000. For the fixed compensation for the financial year 2019, the 2018 AGM has approved a maximum aggregate amount of CHF 3,200,000.

Between January 1, 2018, and September 30, 2018, the Company granted the members of the Executive Management a total of 139,194 SARs with exercise prices between CHF 16.20 and CHF 36.70.

No member of the Executive Management will receive compensation in connection with the Offering.

#### **5. Compensation of former members of the Board and of the Executive Management**

For the compensation of former members of the Board and of the Executive Management for the financial years 2017 and 2016 see the Company's compensation report (at page 89 of the annual report for the financial year 2017 included elsewhere in this Offering Memorandum).

In the financial year 2018, the Company does not expect to pay compensation to former members of the Board and of the Executive Management (other than, for clarity, cash compensation and 21,702 SARs granted to a member of the Executive Management before his resignation during the year 2018).

#### **D. Ownership of Shares and options**

##### **1. Non-executive members of the Board**

The table below sets forth the number of Shares and other purchase positions that each non-executive member of the Board owned as of September 30, 2018. For a description of the stock options and the SARs see “—*Incentive compensation*” in “*The Company and its Business*” beginning on page 57.

<b>Name</b>	<b>Shares</b>	<b>Vested equity rights</b>	<b>Unvested equity rights</b>	<b>Total equity rights</b>
-------------	---------------	-----------------------------	-------------------------------	----------------------------

Elmar Schnee	2,000	21,207	1,870	23,077
Martin Gertsch	38,109	22,026	5,623	27,649
Philipp Gutzwiller	500	14,923	1,317	16,240
Patrick Vink	1,000	18,502	1,385	19,887
<b>Total</b>	<b>41,609</b>	<b>76,658</b>	<b>10,195</b>	<b>86,853</b>

In aggregate, the 41,609 Shares and the 86,853 other purchase positions (stock options and SARs) held by the non-executive Directors correspond to 0.6% and 1.8%, respectively, of the voting rights in the Company (calculated on the basis of the 7,527,479 Shares recorded in the commercial register as of the date of this Offering Memorandum).

The Company expects to make no further SAR grants to the non-executive members of the Board in 2018.

## 2. Executive Management

The table below sets forth the number of Shares and other purchase positions that each member of the Executive Management, including the Delegate of the Board and CEO, owned as of September 30, 2018. For a description of the stock options and the SARs see “—*Incentive compensation*” in “*The Company and its Business*” beginning on page 93.

<b>Name</b>	<b>Shares</b>	<b>Vested equity rights</b>	<b>Unvested equity rights</b>	<b>Total equity rights</b>
Thomas Meier	75,562	39,953	22,833	62,786
Günther Metz	0	24,905	22,252	47,157
Christoph Rentsch	0	35,096	21,803	56,899
Kristina Sjöblom Nygren	0	36,937	0	36,937
Giovanni Stropoli	250	0	20,751	20,751
Oliver Strub	0	25,472	14,498	39,970
<b>Total</b>	<b>75,812</b>	<b>162,363</b>	<b>102,137</b>	<b>264,500</b>

In aggregate, the 75,812 Shares and the 264,500 other purchase positions (stock options and SARs) held by the members of the Executive Management correspond to 1.0% and 3.5%, respectively, of the voting rights in the Company (calculated on the basis of the 7,527,479 Shares recorded in the commercial register as of the date of this Offering Memorandum).

The Company expects to make no further SAR grants to the members of the Executive Management in 2018.

## E. Loans, credits, post-retirement benefits

As of the date of this Offering Memorandum, the Company has not granted any loans and credits to Directors or members of the Executive Management or to any of their related parties.

## F. Agreements regarding compensation with Directors or members of the Executive Management

According to the Articles and in line with the Compensation Ordinance, the Company or companies controlled by it directly or indirectly may enter into agreements with members of the Board relating to their mandate and compensation for a fixed term or for an indefinite term, subject to term of office and the law. The Company or companies controlled by it directly or indirectly may enter into employment agreements with members of the Executive Management for a fixed term of up to one year (renewals being permissible)

or for an indefinite term with a notice period of up to one year. Currently, the employment agreements with members of the Executive Management provide for notice periods between six months and one year.

Post-contractual non-compete undertakings may be entered into for a period of up to one year after termination of employment. The consideration for such non-compete undertaking must not exceed the last total annual compensation of the respective individual.

#### **G. Mandates outside the Company**

As required by the Compensation Ordinance, the Articles limit the number of positions on the supreme governing body of companies other than the Company or its subsidiaries. Generally, Directors may hold up to four such mandates in listed companies and, in addition, up to eight such mandates in non-listed companies, and members of the Executive Management may hold up to two such mandates in listed companies and, in addition, up to four such mandates in non-listed companies. Mandates in associations, charitable organizations, foundations, trusts and the like are not subject to these limitations. However, no Director or member of the Executive Management may hold more than ten such mandates. The same goes for mandates held at the request of the Company or a company controlled by it.

#### **H. Conflicts of interest**

There is no explicit general provision on conflicts of interest in the Swiss Code of Obligations (“CO”). However, the CO requires directors and senior management to safeguard the interests of the company and, in this connection, imposes a duty of loyalty and duty of care on them (see “—*Conflicts of interest*” in “*Description of the Company’s Capital Structure and Shares*” beginning on page 142 for more information).

## **XVI. DESCRIPTION OF THE COMPANY'S CAPITAL STRUCTURE AND SHARES**

*This summary contains certain information in relation to the share capital of the Company and the Shares, as well as a brief description of certain significant provisions of the Articles and Swiss law. This description does not purport to be complete and is qualified in its entirety by the Articles, the relevant excerpt from the commercial register and its underlying documents (Belege) as well as the laws of Switzerland in effect on the date of this Offering Memorandum. Unless otherwise noted, the summary below is based on the versions of those documents that are in effect as of the date of this Offering Memorandum.*

### **A. Capital structure**

#### **1. Issued share capital**

As of the date of this Offering Memorandum, the issued share capital of the Company amounts to CHF 7,527,479 and is divided into 7,527,479 fully paid-up registered Shares (including the Idorsia Shares but excluding the Offered Shares resolved to be created by the extraordinary shareholders' meeting held on December 11, 2018), each with a par value of CHF 1, which form one single class of shares. The General Meeting of Shareholders may at any time convert registered shares into bearer shares (*Inhaberaktien*) and bearer shares into registered shares.

The Shares are fully paid in and non-assessable. Each Share carries one vote.

#### **2. Changes in share capital since 2015**

As at January 1, 2015, the Company's share capital recorded in the commercial register amounted to CHF 4,911,728, divided into 4,911,728 Shares.

On January 28, 2015, the Company amended its articles of association to reflect share issuances out of its conditional share capital. The Company's share capital so recorded in the commercial register amounted to CHF 4,974,492 and was divided into 4,974,492 Shares.

On August 7, 2015, the Company announced that it had completed a sale of 300,000 Shares issued out of its conditional share capital at an average price of CHF 92.38 per Share. The Shares were sold by an independent broker within a period of four days, and the Company received gross proceeds of CHF 27.7 million.

On December 2, 2015, the Company announced that it had completed a private placement of 590,000 Shares out of its authorized share capital via an accelerated bookbuilding process and raised gross proceeds of CHF 54.8 million. The Shares were sold at CHF 93.00 per Share, representing a 4.5% discount on the volume-weighted average price of the Shares on the SIX Swiss Exchange on December 1, 2015. As a result, the Company's share capital recorded in the commercial register increased to CHF 5,564,492, divided into 5,564,492 Shares.

On February 11, 2016, the Company amended its articles of association to reflect share issuances out of its conditional share capital. The Company's share capital so recorded in the commercial register amounted to CHF 6,262,798 and was divided into 6,262,798 Shares.

On March 17, 2017, the Company amended its articles of association to reflect share issuances out of its conditional share capital. The Company's share capital so recorded in the commercial register amounted to CHF 6,279,857 and was divided into 6,279,857 Shares.

On February 21, 2018, the Company issued 238,924 Shares out of its authorized share capital in order to pay the upfront consideration for the license from Polyphor of CHF 6.5 million at an agreed valuation of CHF 27.2053 per Share. In addition, the Company amended its articles of association to reflect share issuances out of its conditional share capital. As a result, the Company's share capital recorded in the commercial register amounted to CHF 6,527,479 and was divided into 6,527,479 Shares.

On November 21, 2018, the Company issued the 1,000,000 Idorsia Shares out of its authorized share capital to Idorsia as consideration for the acquisition of the option for the exclusive sub-license to commercialize ReveraGen's vamorolone (see "*The Issuance of the New Shares*" beginning on page 117 for more information). As a result, the Company's share capital recorded in the commercial register as at the date of this Offering Memorandum amounts to CHF 7,527,479 and is divided into 7,527,479 Shares.

For information on the CHF 60 million convertible Bonds due 2022 see "*—CHF 60 million Senior Unsecured Convertible Bonds 2017-2022*" in "*The Company and its Business*" beginning on page 88.

### 3. Sourcing of the Offered Shares

On December 11, 2018, the shareholders of the Company resolved on an ordinary capital increase of up to CHF 5,000,000 by issuing a maximum of 5,000,000 new shares in the Company with a par value of CHF 1 each (the Offered Shares). The Company intends to use the net proceeds of this Offering to fund the USD 20.0 million payable to Idorsia Pharmaceuticals Ltd as the cash component of the consideration for the acquisition of the option for the exclusive sub-license to commercialize vamorolone (see "*—License and collaboration agreements*" in "*The Company and its Business*" beginning on page 84 for more information) and any net proceeds from the Offering in excess of that amount for general corporate purposes. Upon completion of the Offering, assuming that all 5,000,000 Offered Shares are sold in the Offering, the share capital of the Company will be CHF 12,527,479 and consist of 12,527,479 Shares.

### 4. Authorized share capital

As of the date of this Offering Memorandum, the Company's authorized share capital is set forth in article 3a of the Articles, which reads as follows (convenience translation from the German original):

#### ***“Article 3a – Authorized Share Capital***

*The Board of Directors is authorized at any time until April 11, 2020, to increase the Company's share capital by a maximum of CHF 500,000 by issuing up to 500,000 registered shares with a nominal value of CHF 1.00 each, to be fully paid up. Increases in partial amounts are permitted.*

*The Board of Directors shall define the issue price of new shares, the manner in which they are to be paid up, the time of the issuance of new shares, the conditions for the exercise of the preemptive rights and the beginning of the period of dividend entitlement. In doing so, the Board of Directors may issue new shares through firm underwriting by a bank, bank syndicate or a third party and subsequent offer to the existing shareholders (unless the preemptive rights of the existing shareholders are excluded). The Board of Directors is authorized to permit, restrict or to prohibit trading in the preemptive rights. The Board of Directors may permit preemptive rights that have been granted but not exercised to expire, place them and the shares for which subscription rights have been granted but not exercised, respectively, at market conditions, or use them for other purposes in the interest of the Company.*

*The subscription, the acquisition and each following transfer of the new registered shares are subject to the transfer restrictions set forth in Article 5 of the Articles of Association.*

*The Board of Directors is authorized to restrict or exclude the preemptive rights of the shareholders and to allocate them to third parties if the shares are to be used:*

- (a) for acquisitions of businesses, parts thereof, participations, products, intellectual property rights or licenses, or for investment projects including product development programs, or for the financing or refinancing of such transactions or investment projects by means of a share placement with one or more investors; or*
- (b) for the purposes of participations by strategic partners (including in the case of a public takeover bid) or for expansion of the shareholder constituency in certain investor groups or markets or in connection with the listing of the shares on domestic or foreign stock exchanges, including for the purpose of delivering shares to the participating banks upon exercise of an over-allotment option; or*

- (c) *for the purposes of participations or remuneration of persons or companies that provide services to the company or one of its subsidiaries; or*
- (d) *if the issue price of the new shares is determined in consideration of the market price; or*
- (e) *in order to quickly and flexibly raise equity capital by a share placement that could only be achieved with difficulty or on significantly less favorable terms if the preemptive rights of shareholders were maintained; or*
- (f) *for the defense of an actual, threatened or potential takeover bid that the Board of Directors, upon consultation with an independent financial adviser retained by it, has not recommended to the shareholders because the Board of Directors has not found it to be financially fair to the shareholders.”*

## **5. Conditional share capital**

As of the date of this Offering Memorandum, the Company’s conditional share capital is set forth in articles 3b and 3c of the Articles, which read as follows (convenience translation from the German original):

### **“Article 3b – Conditional Share Capital for Employee Participations**

*The share capital may be increased through the issuance of up to 691,302 registered shares, to be fully paid up with a nominal value of CHF 1.00 each, by up to CHF 691,302 through the direct or indirect issuance of shares, options or respective subscription rights to employees and/or members of the Board of Directors of the Company and its affiliates.*

*The preemptive rights as well as the advance subscription rights of the shareholders of the Company are excluded upon the issue of shares, options or subscription rights in respect thereof. The issue of shares, options or subscription rights in respect thereof is conducted according to one or more participation schemes and/or regulations to be issued by the Board of Directors and taking into consideration Article 4 of the Articles of Association.*

*The new shares directly or indirectly acquired by employees or members of the Board of Directors of the Company or its affiliates as part of an employee participation program and any following transfer of such shares shall be subject to the restrictions of Article 5 of the Articles of Association.*

### **Article 3c – Conditional Share Capital for Financings, Mergers and Acquisitions**

*The share capital of the Company may be increased by up to CHF 930,000 through the issuance of up to 930,000 registered shares, to be fully paid up with a nominal value of CHF 1.00 each, through the exercise or mandatory exercise of options and/or conversion rights granted in connection with bonds, similar debt instruments, notes or other securities or contractual obligations by or of Santhera Pharmaceuticals Holding AG or one of its affiliates, and/or through the exercise of options rights issued by Santhera Pharmaceuticals Holding AG or one of its affiliates (hereinafter collectively, the Financial Instruments). The preemptive rights of the shareholders shall be excluded in connection with the issuance of shares upon the exercise of any Financial Instrument. The respective holders of the Financial Instruments are entitled to subscribe the new shares. The conditions of the Financial Instruments shall be determined by the Board of Directors. The acquisition of registered shares through the exercise of Financial Instruments and the following transfer of the registered shares shall be subject to the transfer restrictions set forth in Article 5 of the articles of association.*

*The Board of Directors shall be authorized to restrict or withdraw the advance subscription rights of the shareholders in connection with the issuance of Financial Instruments,*

- (1) *if the issuance of the Financial Instruments is for purposes of financing or refinancing the acquisition of businesses, parts thereof or participations, cooperations or investments or if it occurs in national or international financial markets (including through private placements);*
- (2) *for purposes of a firm underwriting of the Financial Instruments by a bank or a bank consortium followed by a public offer;*

- (3) *in order to flexibly raise equity capital by a share placement that could only be achieved with difficulty or on significantly less favorable terms if the advance subscription rights of shareholders were maintained.*

*If the advance subscription rights are excluded through a resolution by the Board of Directors, the following shall apply: (i) the Financial Instruments' issue shall be made at the prevailing market conditions (including the standard dilution protection provisions in accordance with market practice), and (ii) the issue of the new shares shall be made pursuant to the relevant conversion or vesting conditions. Conversion or option rights may be exercised during a maximum 10-year period from the date of the respective issue."*

Of the 930'000 Shares that may be issued out of the conditional share capital in article 3c of the Articles, 925,920 Shares have been reserved to cover the conversion rights of the holders of the Bonds (see "*—CHF 60 million Senior Unsecured Convertible Bonds 2017-2022*" in "*The Company and its Business*" beginning on page 88 for more information).

## 6. Share capital as of December 31, 2017

As of December 31, 2017, the Company's issued share capital was 6,288,555 Shares (comprising 6,279,857 Shares recorded in the commercial register and 8,698 Shares issued out of the Company's conditional share capital which had not yet been recorded in the commercial register). As of December 31, 2017, the Company's authorized share capital amounted to CHF 1,500,000, providing for the issuance of up to 1,500,000 Shares; the conditional share capital for employee participations amounted to CHF 691,302, providing for the issuance of up to 691,302 Shares (taking into account the 8,698 Shares issued out of the Company's conditional share capital which had not yet been recorded in the commercial register); and the conditional share capital for financings, mergers and acquisitions amounted to CHF 930,000, providing for the issuance of up to 930,000 Shares.

## 7. Participation certificates and profit sharing certificates

The Company has not issued any non-voting equity securities, such as participation certificates (*Partizipationsscheine*) or profit sharing certificates (*Genussscheine*), and the Company does not have any preference shares (*Vorzugsaktien*).

## 8. Options, warrants and conversion rights

The table below provides a summary of the SARs granted under the Company's share appreciation rights plans (see "*—Share Appreciation Rights Plans (BSARPs and ESARPs)*" in "*The Company and its Business*" beginning on page 93 for more information) and outstanding as of September 30, 2018:

Exercise price range for SARs (in CHF)	Number outstanding as of September 30, 2018	Weighted average remaining contractual life (years)	Number exercisable as of September 30, 2018
from 16.20 to 18.90	67,659	9.58	0
from 36.70 to 38.70	432,318	9.25	0
from 51.75 to 54.85	239,020	8.25	111,142
from 76.50 to 77.80	29,522	8.33	10,154
<b>Total</b>	<b>768,519</b>	<b>9.01</b>	<b>121,296</b>

Between September 30, 2018, and the date of this Offering Memorandum, no further SARs were granted, 26,805 were forfeited, 34,128 became exercisable and none were exercised. The Company expects to make no further SAR grants in 2018.

The table below provides a summary of the stock options granted under the Company's stock option plans (see “—*Stock Option Plans (BSOPs and ESOPs)*” in “*The Company and its Business*” beginning on page 93 for more information) and outstanding as of September 30, 2018:

<b>Exercise price range for options (in CHF)</b>	<b>Number outstanding as of September 30, 2018</b>	<b>Weighted average remaining contractual life (years)</b>	<b>Number exercisable as of September 30, 2018</b>
from 3.89 to 4.53	25,001	4.38	25,001
at 22.25	4,550	5.75	4,550
at 69.30	13,150	7.50	7,400
from 82.10 to 112.60	226,098	6.97	152,277
<b>Total</b>	<b>268,799</b>	<b>6.85</b>	<b>189,228</b>

As the Company has phased out its stock options plans as of July 1, 2016, no further stock options were granted before the date of this Offering Memorandum. Between September 30, 2018, and the date of this Offering Memorandum, 2,212 stock options were forfeited, 4,710 stock options became exercisable, none were expired and none were exercised.

In addition, the Bonds (see “—*CHF 60 million Senior Unsecured Convertible Bonds 2017-2022*” in “*The Company and its Business*” beginning on page 88 for more information) are convertible at any time before the maturity of the Bonds on February 17, 2022, into an aggregate of up to 925,920 Shares at a conversion price of CHF 64.80.

## **9. Treasury shares**

As of September 30, 2018, the Company held 56,104 Shares in treasury in connection with a market making agreement with Kepler Cheuvreux SA.

## **10. Asset transfer and contributions in kind**

Pursuant to an asset transfer agreement (*Vermögensübertragungsvertrag*) dated August 11, 2004, the Company transferred assets in the amount of CHF 1,299,086 and liabilities in the amount of CHF 827,280 to Santhera Pharmaceuticals (Schweiz) GmbH, Liestal, in consideration of one quota in the amount of CHF 124,000 in Santhera Pharmaceuticals (Schweiz) GmbH, Liestal.

On the occasion of the capital increase of June 14, 2005, and pursuant to contribution agreements dated June 14, 2005, the Company took over an aggregate of 149,825 preference shares series (B) in Santhera Pharmaceuticals (Deutschland) AG, in Heidelberg (Germany), at an aggregate price of CHF 656,170, in consideration of 131,234 preference shares series (B) in the Company with a nominal value of CHF 1.00.

On the occasion of the authorized capital increase of September 29, 2009, pursuant to the authorization resolution of April 21, 2009, and pursuant to an agreement on contributions in kind dated September 25, 2009, the Company took over participations at an aggregate price of CHF 105,973, in consideration of which 105,973 registered shares in the Company with a nominal value of CHF 1.00 were issued.

## **11. Cross-shareholdings**

As of the date of this Offering Memorandum, there are no cross-shareholdings of the Company that exceed 5% of the holdings of capital or voting rights on both sides.

## **B. Description of Shares, articles and Swiss law**

### **1. Shares**

The Shares are fully paid-up registered shares with a par value of CHF 1 each. By decision of the shareholders' meeting, registered shares may be converted into bearer shares and vice versa. The Shares rank *pari passu* in all respects with each other, including in respect of entitlements to dividends, liquidation proceeds and pre-emptive rights.

Only persons registered in the Company's share register (*Aktienbuch*) (the "**Share Register**") are recognized as shareholders by the Company.

### **2. Form of the Shares**

The Shares are issued in the form of uncertificated securities (*Wertrechte*) within the meaning of article 973c CO and are maintained as intermediated securities (*Bucheffekten*) within the meaning of the Swiss Federal Intermediated Securities Act of October 3, 2008, as amended (the "**FISA**"; *Bucheffektengesetz*). In accordance with article 973c CO, the Company maintains a register of uncertificated securities (*Wertrechtbuch*).

According to the Articles, the Company may issue its shares in the form of uncertificated securities, single certificates or global certificates. Subject to applicable law, the Company may convert its registered shares from one form into another form at its own cost at any time and without the approval of its shareholders. Shareholders do not have a right to request a conversion of the Shares issued in one form into another form. Each shareholder may, however, at any time request a written confirmation from the Company of the registered shares held by such shareholder, as reflected in the Share Register. Any such confirmation is not a negotiable instrument.

### **3. Transfer of Shares and transfer restrictions**

So long as and to the extent that the Shares are intermediated securities (*Bucheffekten*) within the meaning of the FISA, (i) any transfer of Shares is effected by a corresponding entry in the securities deposit account of a bank or a depository institution, (ii) no Shares can be transferred by way of assignment, (iii) a security interest in any Shares cannot be granted by way of assignment, and (iv) generally, the transfer of or perfection of security over Shares requires action by the custodian with whom the respective shareholder has a custody account.

The Company maintains the Share Register and enters the full name, address and nationality (in the case of legal entities, the company name and registered office) of the shareholders and usufructuaries therein. A person recorded in the Share Register must notify the share registrar of any changes of address. Until such notification occurs, all written communication from the Company to persons entered in the Share Register are deemed to have been validly made if sent to the relevant address recorded in the Share Register.

Any person who acquires Shares may submit a request to the Company to be entered into the Share Register as a shareholder with voting rights, provided such person expressly declares to the Company that it has acquired and holds such Shares in its own name and for its own account.

Any person that does not expressly state in its application to the Company that the relevant Shares were acquired for its own account (any such person, a "**Nominee**") may be entered in the Share Register as a shareholder with voting rights with regard to up to 2% of the share capital recorded in the commercial register, provided that the Nominee has entered into an agreement with the Company regarding its position and information obligations. Beyond such registration limit, the Board may register a Nominee as shareholder with voting rights if such Nominee discloses the name, address and shareholdings of those persons for whose account it holds 2% or more of the share capital recorded in the commercial register. Legal persons and groups with joint legal status that are related to one another through capital ownership, voting

rights, common control or by other means, as well as all natural and legal persons and groups with joint legal status who act in concert in the view of circumventing the transfer restrictions with regard to Nominees (including as a syndicate) are deemed to be one Nominee.

The Board may, after having heard the concerned registered shareholder or Nominee, cancel entries in the Share Register that were based on false or misleading information or, in the case of a Nominee, in the event of a breach of the agreement between the Company and the Nominee, with retroactive effect as of the date of entry. The respective person has to be notified of such cancelation immediately.

The Board has the authority to determine the details and to make the dispositions necessary for compliance with the transfer restrictions set forth in the Articles. In special cases, the Board may grant exceptions from the arrangements regarding nominees. Since January 1, 2016, the Board has granted no exceptions to these rules.

After publication or mailing (as applicable) of the invitation to a general meeting of shareholders until the day after such meeting, no registrations in the share register are made, unless the Board has designated a different cut-off date.

#### **4. Voting rights**

Each Share carries one vote at the general meeting of shareholders of the Company. Voting rights may be exercised only to the extent that a shareholder has been recorded in the Share Register as a shareholder with voting rights (see “—*Transfer of Shares and transfer restrictions*” beginning on page 138). Such recordal must have taken place up to a specific qualifying day (the “**Record Date**”) designated each time by the Board. To the extent that a shareholder is not registered in the Share Register as shareholder with voting rights, it is not entitled to participate in any general meeting of shareholders and exercise any voting rights or related rights. However, such shareholders will be entitled to the economic benefits attached to such Shares, including dividends and preemptive rights (if any).

#### **5. General meeting of shareholders**

Under Swiss law and the Articles, an annual general meeting of shareholders must be held within six months after the end of a company’s financial year. In the case of the Company, the AGM has to be held on or before June 30.

In general meetings of shareholders, except as described below, each shareholder has equal rights, including equal voting rights. According to the Articles, each Share is entitled to one vote (provided that its holder or usufructuary is recorded in the Share Register as a shareholder with voting rights as of the relevant Record Date).

The AGM is convened by the Board or, if necessary, by the Company’s auditors. Extraordinary general meetings may be held when deemed necessary by the Board or the Company’s auditors. Furthermore, extraordinary general meetings must be convened upon resolution of a general meeting of shareholders or upon written request by one or more shareholders who represent an aggregate of at least 10% of the Company’s share capital registered in the commercial register, provided that such request specifies the agenda items and the proposals or, in case of elections, the names of the proposed candidates. One or more shareholders holding Shares with an aggregate nominal value of at least CHF 1,000,000, or representing at least 10% of the Company’s share capital registered in the commercial register, whichever is lower, have the right to request that a specific proposal be put on the agenda for the next general meeting. The Articles require that such request is communicated to the Board at least 60 calendar days prior to the next general meeting. A general meeting of shareholders of the Company is convened at least 20 calendar days prior to such meeting by publishing a notice of the meeting in the Swiss Official Gazette of Commerce (*Schweizerisches Handelsamtsblatt*) (the “**SOGC**”). The Board may designate additional means of publication in individual cases.

There is no provision in the Articles requiring the presence of a quorum for general meetings of shareholders of the Company.

Pursuant to Swiss law and the Articles, shareholders' resolutions generally require the approval of an absolute majority of the votes represented at the general meeting of shareholders (with abstentions, blank or invalid ballots being counted towards the total number of votes whose majority is to be reached) unless otherwise required by Swiss law or the Articles. In the event of a tie, the chair of the general meeting of shareholders has the casting vote. The resolutions requiring the approval of an absolute majority of the votes represented include, *inter alia*, amendments to the Articles (subject to exceptions), the election and removal of the Chairman and the members of the Board, members of the Compensation Committee, the independent voting rights representative and the auditors, approval of the annual report and the financial statements, approval of dividends (if any), approval of the aggregate amounts of compensation of the members of the Board and the Executive Management, releasing the members of the Board and the Executive Management from liability for matters disclosed to the general meeting of shareholders, and ordering an independent investigation into specific matters proposed to the general meeting (*Sonderprüfung*).

A resolution passed at a general meeting of shareholders with a qualified majority of at least two-thirds of the votes represented and the absolute majority of the nominal share capital represented at such meeting (a “**Qualified Majority**”) is required by law and/or the Articles for: (i) modifications of the Company's purpose; (ii) the creation of shares with preferential voting rights; (iii) restrictions of the transferability of registered shares and the easing or lifting of such restrictions; (iv) an authorized or conditional capital increase; (v) a share capital increase by conversion of equity surplus, against contributions in kind or for purposes of an acquisition of assets, or the granting of special benefits; (vi) the limitation or exclusion of preemptive rights of shareholders; (vii) the relocation of the registered office of the Company; and (viii) the dissolution of the Company. The Qualified Majority requirement and, in some instances, other qualified majority requirements, apply by operation of law to a merger (*Fusion*), demerger (*Spaltung*) or conversion (*Umwandlung*) of the Company. The introduction or abolition of any provision of the Articles providing for a higher majority requirement than is prescribed by law must be adopted by such majority.

## **6. Representation of shareholders at general meetings of shareholders**

Shareholders of the Company may elect to be represented by proxy at general meetings of shareholders by another shareholder with voting rights, by their legal representative(s), or by the independent voting rights representative. All shares held by a shareholder may only be represented by one such person.

The Compensation Ordinance requires that one or several independent voting rights representatives be elected by the general meeting of shareholders on an individual basis for a term ending at the next annual general meeting. Re-election is permitted. Shareholders are able to electronically grant proxies and instruct the independent voting rights representative on both (i) agenda items included in the invitation to the general meeting of shareholders and (ii) new motions that were not disclosed in the invitation to the general meeting. The independent voting rights representative is bound by the voting instructions of the respective shareholders; if the independent voting rights representative has not received voting instructions, he is required to abstain from voting.

## **7. Preemptive rights and advance subscription rights**

Under Swiss law, any share issue, whether for cash or non-cash consideration, is subject to the prior approval of the shareholders at a general meeting of shareholders. Shareholders have certain preemptive rights (*Bezugsrechte*) to subscribe for new issues of shares and advance subscription rights (*Vorwegzeichnungsrechte*) to subscribe for convertible or warrant-bearing bonds or other financial instruments in proportion to the nominal amount of shares held. A resolution adopted at a general meeting of shareholders by a Qualified Majority may limit or exclude preemptive rights in certain limited circumstances. According to the Articles, the Board is authorized to limit or exclude preemptive rights and advance subscription rights in connection with share issues out of its authorized and conditional share capital (see “—*Authorized share capital*” beginning on page 134 and “—*Conditional share capital*” beginning on page 135).

## 8. Dividends and other distributions

Under Swiss law, dividends may be paid out only if the Company has sufficient distributable profits from previous financial years or if the Company has freely distributable reserves, each as presented on the Company's annual statutory standalone balance sheet. Payments out of the Company's share capital (*i.e.*, the aggregate nominal value of the Company's issued share capital) in the form of dividends are not allowed. However, payments out of the share capital may be made by way of a capital reduction. Such a capital reduction requires the approval of an absolute majority of the votes represented at the Company's general meeting of shareholders. A special audit report is required which confirms that claims of the Company's creditors remain fully covered after the capital reduction. Upon approval by the general meeting of shareholders, the Board must give public notice of the respective capital reduction resolution in the SOGC three times and notify creditors that they may request, within two months after the third publication, satisfaction of or security for their claims. Qualifying additional paid-in capital may only be paid out to shareholders (to the extent permissible under the CO) upon the affirmative vote of an absolute majority of the votes represented at the general meeting of shareholders. The Board may propose to shareholders that a dividend or other distribution be paid but cannot itself authorize the distribution.

Under the CO, if the Company's general reserves amount to less than 20% of its share capital recorded in the commercial register (*i.e.*, 20% of the aggregate nominal value of the Company's registered capital), at least 5% of the Company's annual profit must be retained as general reserves. The CO permits the Company to accrue additional general reserves. In addition, the Company is required to present the amount of the purchase price of Shares repurchased as a negative item in its equity on its annual standalone statutory balance sheet, and such amount may not be used for dividends or subsequent repurchases.

The Company must prepare a separate standalone "statutory" balance sheet for the purpose of, among other things, determining the amounts available for distributions to shareholders, including by way of a distribution of dividends. The Company's auditors must confirm that a proposal made by the Board to shareholders regarding the appropriation of the Company's unappropriated profit and/or a distribution complies with the requirements of the CO and the Articles. Dividends are usually due and payable shortly after the shareholders have passed a resolution approving the payment, but shareholders may also resolve in the general meeting of shareholders to pay dividends in quarterly or other instalments. The Articles provide that dividends that have not been claimed within five years after the due date will become the property of the Company and be allocated to the general reserves. Dividends paid are subject to Swiss federal withholding tax (except if paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*)), all or part of which can potentially be reclaimed under the relevant tax rules in Switzerland or double taxation treaties entered into between Switzerland and other countries. Distributions of cash or distributions in kind that are based upon a capital reduction are not subject to Swiss federal withholding tax. See "*—Swiss taxation*" in "*Taxation*" beginning on page 156 for a summary of certain Swiss tax consequences regarding dividends paid to holders of the Shares and distributions paid on the Shares that are based upon a capital reduction. For information on the Company's dividend policy, see "*Dividends and Other Distributions*" beginning on page 38.

Dividends, if declared by the Company, will be declared and paid in Swiss francs.

## 9. Ordinary capital increase, authorized and conditional share capital

Pursuant to Swiss law and the Articles, the share capital of a company may be increased against cash contributions by a resolution passed at a general meeting of shareholders by an absolute majority of the votes represented. A capital increase against contributions in kind or at the exclusion of the preemptive rights (*Bezugsrechte*) of the shareholders or by way of a reclassification of reserves into share capital requires a resolution passed by a Qualified Majority. Furthermore, under the CO, the shareholders of a company may authorize, by passing a resolution with a Qualified Majority, the issuance of shares in the form of:

- (a) authorized share capital (*genehmigtes Kapital*) up to a specified aggregate nominal amount, but not more than 50% of the existing share capital, to be utilized by the board of directors within a period not exceeding two years from the approval given in the general meeting of shareholders; and/or

- (b) conditional share capital (*bedingtes Kapital*) up to a specified aggregate nominal amount, but not more than 50% of the existing share capital, for the purpose of issuing shares, *inter alia*, (i) to grant conversion rights or warrants to holders of convertible bonds, or (ii) to grant rights to employees of a company or affiliated companies to subscribe for new shares.

#### **10. Ownership of Shares by non-Swiss persons**

Except for the limitation on voting rights described above applicable to shareholders generally, and subject to government sanctions (see next subsection), there is no limitation under Swiss law or the Articles on the right of non-Swiss residents or nationals to own Shares or to exercise voting rights attached to the Shares applicable to stock corporations of the type of and conducting a business as the Company.

#### **11. Foreign investment and exchange control regulations in Switzerland**

Other than in connection with government sanctions imposed on certain persons from the Republic of Iraq, the Islamic Republic of Iran, Lebanon, Yemen, Libya, Sudan, the Republic of South Sudan, Burundi, the Democratic Republic of Congo, Somalia, Guinea-Bissau, Eritrea, Syria, Myanmar (Burma), Zimbabwe, Belarus, Guinea, and the Democratic People's Republic of Korea (North Korea), the Central African Republic, the Republic of Mali and Venezuela, persons and organizations with connections to Osama bin Laden, the "Al-Qaeda" group or the Taliban, certain persons in connection with the assassination of Rafik Hariri, and certain measures in connection with the prevention of circumvention of international sanctions in connection with the situation in the Ukraine, there are currently no government laws, decrees or regulations in Switzerland that restrict the export or import of capital, including, but not limited to, Swiss foreign exchange controls on the payment of dividends, interest or liquidation proceeds, if any, to non-resident holders of the Shares.

#### **12. Borrowing power**

Neither Swiss law nor the Articles generally restrict the Company's power to borrow and to raise funds. The decision to borrow funds is made or delegated by the Board and no shareholders' resolution is required in relation to any such borrowing. However, the terms and conditions of the Bonds restrict the Company's ability to incur additional indebtedness in that the Company and certain of its subsidiaries may not issue any secured marketable debt instruments or incur any secured financial debt (including bank debt) exceeding CHF 10 million in the aggregate (subject to exceptions) unless the Bonds are secured equally and rateably or agreed by the Paying and Conversion Agent under the Bonds.

#### **13. Conflicts of interest**

There is no explicit general provision on conflicts of interest in the CO. However, the CO requires directors and senior management to safeguard the interests of the company and, in this connection, imposes a duty of loyalty and duty of care on the company's directors and officers. This rule is generally understood to disqualify directors and senior officers of a company from participating in decisions that directly affect them. A company's directors and officers are personally liable to the company for a breach of this rule. In addition, the CO contains provisions under which directors and all persons engaged in the management of a company are liable to the company, each shareholder and the company's creditors for losses caused by an intentional or negligent breach of their duties. Furthermore, the CO contains a provision under which payments made to any shareholders or directors of a company or any person associated with any such shareholder or director, other than payments made at arm's length, must be repaid to the Company if such shareholder or director was acting in bad faith. In addition, if, in connection with entering into a contract (except relating to daily business matters of up to CHF 1,000), a company is represented by the person with whom it is entering into the contract, such contract must be in writing.

For information on the Swiss law rules governing compensation of the members of the Board and of the Executive Management, including the disclosure of such compensation, see "*Legal framework and its implementation in the Articles*" in "*Board of Directors and Executive Management*" beginning on page 127. For information on the reporting of transactions in equity securities by members of the Board and of

the Executive Management and, in some cases, their related parties, see “—*Directive on the Disclosure of Management Transactions*” in “*SIX Swiss Exchange*” beginning on page 146. Furthermore, the Directive on Information Relating to Corporate Governance issued by the SIX Swiss Exchange also addresses conflict of interest issues, see “—*Corporate Governance Directive*” in “*SIX Swiss Exchange*” beginning on page 146.

#### **14. Purchases of own shares**

Swiss law limits the right of a company to purchase and hold its own shares in treasury. A company or its subsidiaries may purchase shares of the company only if and to the extent that (a) such company has freely distributable reserves in the amount of the purchase price, and (b) the aggregate nominal value of all shares held by the company does not exceed 10% of the company’s share capital (20% in specific circumstances). Furthermore, the company must present the acquired shares on its statutory balance sheet as a negative item in its equity. For tax implications in case of cancellation of own shares or exceeding thresholds, see “—*Swiss taxation*” in “*Taxation*” beginning on page 156.

Shares held by the Company or its subsidiaries do not carry any rights to vote at general meetings of shareholders, but are entitled to the economic benefits attached to such Shares generally, including dividends and preemptive rights (if any).

Selective share repurchases are only permitted under certain circumstances. In particular, publicly announced repurchases of listed shares are subject to certain restrictions promulgated by the Swiss Takeover Board (the regulatory body for takeover bids in Switzerland) under the Federal Act on Financial Market Infrastructures and Market Conduct in Securities and Derivatives Trading of June 19, 2015 (the “**FMIA**”), and the implementing ordinances enacted thereunder. Within these limitations, as is customary for Swiss companies, the Company may purchase and sell its own Shares from time to time.

#### **15. Duration and liquidation**

The Articles do not limit the Company’s duration. Under Swiss law, the Company may be dissolved at any time, by way of liquidation or by way of a merger in accordance with the Merger Act, based on a resolution of a general meeting of shareholders, which must be passed by a Qualified Majority. Dissolution and liquidation by court order is possible if, among other things, (a) the Company becomes bankrupt or (b) shareholders holding at least 10% of the Company’s share capital so request for important reasons. Under Swiss law, any surplus arising out of a liquidation (after the settlement of all claims of all creditors) is distributed in proportion to the paid-up nominal value of shares held. This surplus is subject to Swiss federal withholding tax, except if paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*). See “—*Swiss taxation*” in “*Taxation*” beginning on page 156.

#### **16. Reporting and disclosure of major shareholdings**

Under the FMIA and its implementing ordinances, persons who directly, indirectly or in concert with other parties acquire or dispose of Shares or are granted the power to exercise the voting rights attached to Shares at their own discretion (“delegated voting rights”) or acquire or dispose of purchase or sale rights relating to Shares, and thereby reach, exceed or fall below a threshold of 3%, 5%, 10%, 15%, 20%, 25%, 33 1/3%, 50% or 66 2/3% of the Company’s voting rights (whether exercisable or not), must report such acquisition or disposal to the Company and the SIX Swiss Exchange in writing within four trading days. Within two trading days after the receipt of such notification, the Company must publish such information through SIX Swiss Exchange’s electronic reporting and publishing platform. For purposes of calculating whether a threshold has been reached or crossed, shares, delegated voting rights and acquisition rights or obligations (“**Purchase Positions**”) on the one hand and sale rights or obligations (“**Sale Positions**”) on the other hand may not be netted. Rather, the Purchase Positions and the Sale Positions must be accounted for separately and may each trigger disclosure obligations if the respective positions reach one of the thresholds. In addition, actual share ownership and delegated voting rights must be reported separately from other Purchase Positions if it reaches one of the thresholds.

Furthermore, under the CO, the Company must disclose the identity of shareholders and shareholder groups acting in concert who hold more than 5% of the Company's voting rights in the notes to the financial statements as published in the Company's annual report.

#### **17. Opting out of mandatory tender offer rules**

Pursuant to the FMIA, any person that acquires shares of a company whose shares are listed on a Swiss stock exchange, whether directly or indirectly or acting in concert with third parties, and, as a result, exceeds the threshold of 33 1/3% of the voting rights (whether exercisable or not) of such company, must submit a public tender offer to acquire 100% of the listed equity securities of such company, subject to certain exceptions. The FMIA allows companies to waive this requirement or raise the relevant threshold to up to 49% ("opting-out" and "opting-up", respectively) in their articles of association.

The Company has opted out of the mandatory tender offer rules in its Articles. The shareholders' meeting may resolve to opt in again.

#### **18. Cancellation of remaining equity securities**

Under the FMIA, any offeror who has made a public tender offer for equity securities of a listed Swiss company, and who, as a result of such offer, holds more than 98% of the voting rights of such company, may petition the court to cancel such company's remaining equity securities. The petition must be filed against the target company within three months after the expiration of the offer period. The remaining shareholders of the target company may join in the proceedings. If the court orders cancellation of the remaining equity securities, the target company must reissue and deliver such equity securities to the offeror against payment of the offer consideration for the benefit of the holders of the canceled equity securities.

#### **19. Squeeze-out merger**

The Merger Act allows a squeeze-out of minority shareholders by way of a squeeze-out merger. With the approval of the holders of at least 90% of all shares of the target company, the target company may be merged into another company and the minority shareholders of the target company may be compensated in cash or other consideration (e.g., securities from another company) instead of receiving shares in the surviving company. It is unclear and controversial whether the denominator of the 90% approval threshold is the total number of all issued shares of the target company or the total number of shares that are entitled to be voted.

#### **20. Shareholders' inspection rights**

A shareholder may, upon application to the Company, inspect the minutes of a general meeting of shareholders. In accordance with Swiss law, the Company makes its annual report and the auditors' report available for inspection by shareholders at its registered address at least 20 days prior to each annual general meeting of shareholders. Any shareholder may request a copy of these reports in advance of or after the annual general meeting. In addition, at a general meeting of shareholders, a shareholder may request information from the Board concerning the business and operations of the Company and may request information from the Company's auditors concerning the performance and results of their audit of the financial statements. The Company may refuse to provide such information to a shareholder if, in its opinion, the disclosure of the requested information would reveal confidential business secrets or infringe other protected interests of the Company.

#### **21. Shareholders' rights to bring derivative actions**

According to the CO, an individual shareholder may bring an action, in its own name and for the benefit of the Company, against the Company's directors, officers or liquidators for the recovery of any losses the Company has suffered as a result of the intentional or negligent breach by such directors, officers or liquidators of their duties.

## **XVII.SIX SWISS EXCHANGE**

### **A. General information**

Since the first listing of the Shares on the SIX Swiss Exchange, and for so long as any Shares remain listed on the SIX Swiss Exchange, the Company has been and will be subject to the Listing Rules and any additional regulations enacted by the SIX Swiss Exchange from time to time.

The SIX Swiss Exchange (formerly known as the SWX Swiss Exchange AG) was founded in 1993 as the successor of the local stock exchanges in Zurich, Basel and Geneva. Full electronic trading in foreign equities and derivatives began in 1995. In 1996, the SIX Swiss Exchange introduced full electronic trading in Swiss equities, derivatives and bonds. In 2008, the SWX Swiss Exchange AG changed its name to SIX Swiss Exchange AG. The SIX Swiss Exchange has a single regulatory standard for the listing of equity securities, the Standard for Equity Securities, and two main sub-standards (“International Reporting” and “Swiss Reporting”). The currently issued Shares are, and the New Shares will be, listed according to the International Reporting Standard of the SIX Swiss Exchange.

In 2017, the aggregate trading volume of the SIX Swiss Exchange for Swiss and foreign equity (on, off and dark order book) was CHF 1.048 trillion. As of November 26, 2018, 146 issuers (of equity securities) were listed according to the International Reporting Standard of the SIX Swiss Exchange (source: [https://www.six-swiss-exchange.com/shares/companies/issuer\\_list\\_en.html](https://www.six-swiss-exchange.com/shares/companies/issuer_list_en.html)).

### **B. General rules on securities trading**

Trading on the SIX Swiss Exchange occurs through a fully integrated trading system covering the entire process from trade order through settlement. Trading in equity securities begins each business day at 9:00 am and continues until 5:30 pm Swiss time. After the close of exchange trading, new orders can be entered or deleted until 10:00 pm Swiss time. From 6:00 am Swiss time new entries and enquiries can be made until 9:00 am Swiss time. The system is not available between 10:00 pm and 6:00 am Swiss time. For the opening phase (starting at 9:00 am Swiss time), the system closes the order book and starts opening procedures, establishes the opening prices and determines orders to be executed according to the matching rules. Closing auctions are held to determine the daily closing price for all equity securities traded on the SIX Swiss Exchange. At the start of the closing auction, the status of all equity order books changes from permanent trading to auction. The auction itself consists of a pre-opening period and the actual auction according to rules that are similar to the opening procedure.

Transactions take place through the automatic matching of orders. Each valid order of at least a round lot is entered and listed according to the price limit. A round lot of the shares is expected to consist of one share. In general, market orders (orders placed at best price) are executed first, followed by limit orders (orders placed at a price limit), provided that if several orders are listed at the same price, they are executed according to the time of entry. The SIX Swiss Exchange may provide for a duty to trade on the SIX Swiss Exchange in individual market segments. This duty obliges the participant, during trading hours, to execute orders on the order book only. The duty to trade on the SIX Swiss Exchange for Mid-/Small-Cap equity shares does apply to (i) orders with a market price of CHF 200,000 or more, (ii) collective orders, if the market price of the order is CHF 1,000,000 or more, or (iii) portfolio orders. Members of the SIX Swiss Exchange must observe the principle of best execution for any off-exchange transaction during the trading period. Transactions in shares effected by or through members of the SIX Swiss Exchange are subject to a stock exchange levy. This levy includes the reporting fee and is payable per trade and participant. The fee is defined individually for each trading segment.

Banks and broker-dealers doing business in Switzerland are required to report all transactions in listed securities traded on the SIX Swiss Exchange. Reporting occurs automatically for on order book transactions. Off-order book transactions during trading hours must be reported to the SIX Swiss Exchange within 30 minutes. Transaction information is collected, processed and immediately distributed by the SIX Swiss Exchange. Transactions outside trading hours must be reported no later than the next opening. The SIX

Swiss Exchange distributes a comprehensive range of information through various publications, including in particular the Swiss Market Feed. The Swiss Market Feed supplies SIX Swiss Exchange data in real time to all subscribers as well as to other information providers such as SIX Financial Information Ltd and Reuters.

A quotation may be suspended by the SIX Swiss Exchange if large price fluctuations are observed, or if important, price-sensitive information is about to be disclosed, or in other situations that might endanger fair and orderly trading. Surveillance and monitoring is the responsibility of the SIX Swiss Exchange as the organizer of the market. The aim of such self-regulation is to ensure transparency, fair trading and an orderly market.

**C. Clearing, payment and settlement**

Clearing and settlement of securities listed on the SIX Swiss Exchange, including of the Shares, is made through SIS. Delivery against payment of exchange transactions usually occurs two trading days after the trade date.

**D. Corporate Governance Directive**

The Corporate Governance Directive requires issuers of equity securities listed on the SIX Swiss Exchange to disclose in their annual report, among other things, certain important information on the management and control mechanisms at the highest corporate level or to give specific reasons why this information is not disclosed.

**E. Directive on the Disclosure of Management Transactions**

The Directive on the Disclosure of Management Transactions issued by the SIX Swiss Exchange (the “DMT”) requires issuers whose equity securities have their primary listing on the SIX Swiss Exchange to ensure that members of their board of directors and senior management disclose transactions they have made in the securities of their own company. Under the DMT, the relevant persons must disclose any such transaction to the issuer, and the issuer must forward such information to the SIX Swiss Exchange. Such transactions are subsequently published on a no names basis on the SIX Swiss Exchange’s website.

## **XVIII. OFFERING AND SALE**

### **A. Offering**

This Offering relates to the Offered Shares only. The Idorsia Shares have already been sold and are not being offered pursuant to this Offering Memorandum. This Offering consists of (i) a public offering in Switzerland, (ii) private placements in certain jurisdictions outside the United States and Switzerland, in accordance with applicable securities laws and in reliance on Regulation S, and on the basis of exemptions provided by the Prospectus Directive, in accordance with applicable securities laws, and (iii) private placements within the United States to QIBs in reliance upon the exemption from the registration requirements of the Securities Act provided by Rule 144A. For a description of certain restrictions regarding the Offering and the sale of the Offered Shares, see “*Selling and Transfer Restrictions*” beginning on page 151.

The Company intends to complete the Offering as long as the net proceeds exceed the CHF equivalent of USD 20 million.

### **B. Placement of Offered Shares**

Under the terms and subject to the conditions contained in an accelerated book-build agreement, dated on or around the date of this Offering Memorandum (the “**Accelerated Book-Build Agreement**”), the Managers agree to purchase from the Company up to 5,000,000 Offered Shares. Based on 12,527,479 Shares to be recorded in the commercial register as of the First Day of Trading assuming that all 5,000,000 Offered Shares are sold in the Offering, the Managers will control 39.91% of the company's voting rights as of the First Day of Trading.

The Accelerated Book-Build Agreement provides that the Managers will, subject to certain conditions, purchase the Offered Shares from the Company, each at the Offer Price, less commissions, which may be deducted from the proceeds of the Offering. On the same day, the Managers will immediately allocate and sell the respective Offered Shares to the initial purchasers. Fees will be paid to the Managers for their services rendered pursuant to the Accelerated Book-Build Agreement. The Swiss federal issue stamp duty (*Emissionsabgabe*) on the issuance of the newly issued Offered Shares will be borne by the Company.

The Accelerated Book-Build Agreement provides that the obligations of the Managers are subject to certain conditions precedent, including, without limitation, (i) the absence of a material adverse change in the business of the Company, (ii) the execution of an offer size and pricing supplement and the delivery of the pricing supplement, and (iii) the making of necessary filings and the receipt of necessary approvals in connection with the Offering. The Managers have the right to terminate the Accelerated Book-Build Agreement upon the occurrence of certain events. If the right to terminate the Accelerated Book-Build Agreement is exercised, the Offering will lapse and any previously purported allocation and purchase of Offered Shares will be deemed not to have been made.

In connection with the Accelerated Book-Build Agreement, the Company has made certain representations and warranties and agreed, subject to certain limitations and exemptions, to indemnify the Managers against certain liabilities in connection with the Offering.

The Company has agreed to pay, among others, the costs associated with the distribution of this Offering Memorandum, certain legal expenses of the Company and the Managers, costs of accountants and other advisors retained by the Company and the Managers, costs associated with the delivery of the Offered Shares and all fees and expenses incurred with the listing of the Shares on the SIX Swiss Exchange.

### **C. Lock-up arrangements**

The Company has agreed with the Managers that, during the period commencing on the date of the Accelerated Book-Build Agreement and ending 90 days after the First Day of Trading, and subject to certain

exceptions, the Company and its subsidiaries shall not, without the prior written consent of the Global Coordinator and Bookrunner, acting on behalf of the Managers, (i) issue, offer, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, pledge, grant instruction rights (*Weisungsrechte*) pursuant to art. 25 FISA or otherwise transfer or dispose of (or publicly announce any such issuance, offer, sale or disposal), directly or indirectly, or file a registration statement under any securities regulation relating to, any Shares or any securities representing or convertible into or exchangeable or exercisable for Shares or warrants or other rights to purchase any Shares, (ii) enter into any swap, hedge or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the Shares, or (iii) announce its intention to do any of the foregoing whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Shares or other securities, in cash or otherwise. The foregoing lock-up obligation shall not apply to (i) any options, Shares, stock appreciation rights, performance shares, restricted share units or other equity-linked instruments granted to (or for the benefit of) the Group's employees, management, directors and advisors pursuant to its existing participation or similar plans (the "**Plans**"), (ii) Shares issued upon the exercise of awards granted pursuant to such Plans, issued to a subsidiary of the Company to serve as reserve for such exercises, or issued upon the exercise of conversion rights under the Company's CHF 60 million Senior Unsecured Convertible Bonds 2017-2022, (iii) Shares issued or rights to receive Shares granted as consideration or in-licensing of compounds, product candidates, technology or businesses, provided that the market value of such Shares (or the Shares underlying such rights, as applicable) does not exceed CHF 30 million in aggregate and the acquiring entity enters into the same lock-up undertaking with the Managers, (iv) Shares or other securities of the Issuer acquired in open market transactions after the First Day of Trading, (v) transactions among Group companies, provided that the Company shall give prior notice to the Global Coordinator and Bookrunner, acting on behalf of the Managers, and any Group company acquiring Shares shall enter into the same lock-up undertaking or (vi) any corporate actions in connection with a takeover offer, capital reorganization, legal merger, corporate restructuring, split-up or similar transaction or process, in each case involving the Company.

None of the members of the Board or Executive Management nor any other shareholders of the Company (other than the Company itself) will be subject to any lock-up obligations in connection with the Offering.

#### **D. Share capital upon completion the Offering**

On December 11, 2018, the shareholders of the Company resolved on an ordinary capital increase of up to CHF 5,000,000 by issuing a maximum of 5,000,000 new shares in the Company with a par value of CHF 1 each (the Offered Shares).

Upon completion of the Offering, assuming that all 5,000,000 Offered Shares are sold in the Offering, the share capital of the Company will be CHF 12,527,479 and consist of 12,527,479 Shares. In this case, the 5,000,000 Offered Shares will, in aggregate, represent 39.91% of the share capital of the Company upon completion of the Offering.

For more information, see "*—Sourcing of the Offered Shares*" in "*Description of the Company's Capital Structure and Shares*" beginning on page 134.

#### **E. Allocation**

The Managers have agreed to observe the allocation directives on the New Issues Market issued by the Swiss Bankers Association on March 29, 2004, which entered into force on January 1, 2005.

#### **F. Offer period**

The offer period is expected to be from December 12, 2018, to December 13, 2018, at 4:00 p.m. (CET).

The Company and the Managers reserve the right to extend or shorten the offer period or terminate the Offering, without any prior notice, at any time and for any reason.

## **G. Offer Price**

The Offer Price and the final number of Shares will be determined following an accelerated bookbuilding process. The Company expects to publish the Offer Price and the final number of Shares sold in the Offering by electronic media, by press release and a pricing supplement to this Offering Memorandum on or around December 14, 2018.

## **H. Listing and trading**

Application has been made and approval has been given subject to certain conditions to list the Offered Shares on the SIX Swiss Exchange according to the International Reporting Standard.

The Company expects that the Offered Shares will be listed, and that the Offered Shares will be tradeable, on the SIX Swiss Exchange according to the International Reporting Standard as from or around December 18, 2018.

## **I. Payment and settlement (Closing)**

The Shares are cleared through SIS. It is expected that delivery of the Offered Shares against payment of the Offer Price will be made in book-entry form through the facilities of SIS on or around December 18, 2018. If the right to terminate the Accelerated Book-Build Agreement is exercised, the Offering will lapse and any previously purported allocation and purchase of Offered Shares will be deemed not to have been made.

## **J. Form of Shares**

The Offered Shares will be issued as uncertificated securities (*Wertrechte*) within the meaning of article 973c CO. The Offered Shares will be registered in the main register (*Hauptregister*) maintained by SIS and credited to the securities account of each purchaser, and thus will constitute book entry securities (*Bucheffekten*) within the meaning of the FISA. Shareholders may request from the Company a confirmation relating to their shareholdings in the Company.

## **K. Voting rights**

Each Share carries one vote. Regarding transfers of Shares and transfer restrictions, see “—*Transfer of Shares and transfer restrictions*” in “*Description of the Company’s Capital Structure and Shares*” beginning on page 138.

## **L. Notification/amendments or changes**

Any notices containing or announcing amendments or changes to the terms of the Offering or to this Offering Memorandum will be announced through electronic media. Notices required under the Listing Rules will be published in electronic form on the website of the SIX Swiss Exchange (currently: [https://www.six-group.com/exchanges/news/official\\_notices/search\\_en.html](https://www.six-group.com/exchanges/news/official_notices/search_en.html)). Changes so notified will be deemed to constitute an amendment or supplement of this Offering Memorandum.

The Offer Price and the final number of Offered Shares sold in the Offering will be published via electronic media, by press release and in a pricing supplement to this Offering Memorandum on or around December 14, 2018.

**M. Dividends**

The Offered Shares will be entitled to dividends as from the date of their registration in the commercial register. For more information, see “*Dividends and Other Distributions*” beginning on page 38. Dividends paid on the Shares are subject to Swiss federal withholding tax, except if paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*) (see “—*Swiss taxation*” in “*Taxation*” beginning on page 151).

**N. Listing agent**

Homburger AG, as recognized representative according to article 43 of the Listing Rules, has filed the application for the listing of the Shares on the SIX Swiss Exchange according to the International Reporting Standard on behalf of the Company.

**O. Other relationships of the Managers**

The Managers or its affiliates have provided and may continue to provide investment banking, financing and other services to the Company or its subsidiaries in the ordinary course of their respective businesses for which they have been or will be paid customary fees and may have come to have interests that may not be aligned or could potentially conflict with the interests of the Company and/or its shareholders. In addition, the Managers and any of their respective affiliates may have held and in the future may hold the Company’s securities for investment purposes in the ordinary course of their respective businesses.

## **XIX. SELLING AND TRANSFER RESTRICTIONS**

### **A. General offering restrictions**

No action has been, or will be, taken in any jurisdiction other than Switzerland where action for that purpose is required, which would permit a public offering of the Offered Shares or the possession, circulation or distribution of this Offering Memorandum or any material relating to the Offered Shares. Accordingly, the Offered Shares may not be offered or sold, directly or indirectly, and neither this Offering Memorandum nor any other offering material or advertisements in connection with the Offered Shares may be distributed or published, in or from any country or jurisdiction, except under circumstances that will result in compliance with any applicable rules and regulations of any such country or jurisdiction. The Idorsia Shares have already been sold and are not being offered pursuant to this Offering Memorandum.

The Company has represented and agreed that it has not made, and will not make, any application for listing of the Shares on any exchange outside Switzerland.

### **B. United States**

The Offered Shares have not been and will not be registered under the Securities Act or under the securities laws of any state of the United States. The Offered Shares may not be offered, sold or delivered, directly or indirectly, within or into the United States, except pursuant to an exemption from, or in transactions not subject to, the registration and reporting requirements of the U.S. securities laws and in compliance with all other applicable provisions of U.S. law. Accordingly, the Offered Shares are being offered and sold outside the United States only pursuant to Regulation S, and within the United States only to QIBs pursuant to Rule 144A.

Because of the following restrictions, prospective investors are advised to consult legal counsel prior to making any offer, resale, pledge or other transfer of the Offered Shares.

Each purchaser of Offered Shares in the United States will be deemed to have represented and agreed as follows (terms used herein that are defined in Rule 144A or Regulation S are used herein as defined therein):

- (a) The purchaser is authorized to consummate the purchase of the Offered Shares in compliance with all applicable laws and regulations.
- (b) The purchaser is, and at the time of the purchase of any Offered Shares will be, a “qualified institutional buyer” within the meaning of Rule 144A.
- (c) The purchaser understands and acknowledges that the Offered Shares have not been, or will not be, registered under the Securities Act, that sellers of the Offered Shares may be relying on the exemption from the registration requirements of Section 5 of the Securities Act provided by Rule 144A thereunder and that the Offered Shares may not be offered or sold, directly or indirectly, in the United States, other than in accordance with subclause (d) below.
- (d) The purchaser is purchasing the Offered Shares, (i) for its own account or (ii) for the account of one or more QIBs for which it is acting as duly authorized fiduciary or agent with sole investment discretion with respect to each such account and with full authority to make the acknowledgments, representations and agreements herein with respect to each such account (in which case it hereby makes such acknowledgments, representations and agreements on behalf of such QIBs as well), in each case for investment and not with a view to any resale or distribution of any Offered Shares.
- (e) The purchaser understands and agrees that offers and sales of Offered Shares are being made in the United States only to QIBs, in each case pursuant to Rule 144A, and that if in the future it or any such other QIB for which it is acting, as described in subclause (c) above, or any other fiduciary or agent representing such investor decides to offer, sell, deliver, hypothecate or otherwise transfer any Offered Shares, it or any such other QIB and any such fiduciary or agent will do so only (i) pursuant to an effective registration statement under the Securities Act, (ii) to a QIB in a transaction meeting

the requirements of Rule 144A, (iii) outside the United States in an “offshore transaction” pursuant to Rule 903 or Rule 904 under Regulation S (and not in a pre-arranged transaction resulting in the resale of such Offered Shares into the United States), (iv) pursuant to any other exemption from the registration requirements of the U.S. Securities Act, subject to the receipt by the Company of an opinion of counsel or such other evidence that the Company may reasonably require that such sale or transfer is in compliance with the U.S. Securities Act, or (v) in accordance with Rule 144 and, in each case, in accordance with any applicable securities laws of any state or territory of the United States and of any other jurisdiction. The purchaser understands that no representation can be made as to the availability of the exemption provided by Rule 144 for the resale of the Offered Shares.

- (f) The purchaser understands that the Offered Shares are “restricted securities” within the meaning of Rule 144(a)(3) and, for so long as the Offered Shares are “restricted securities” within the meaning of Rule 144(a)(3), no such Offered Shares may be deposited into any American depositary receipt facility established or maintained by a depositary bank, other than a restricted depositary receipt facility and that such Offered Shares will not settle or trade through the facilities of The Depository Trust Company (“DTC”) or any other U.S. clearing system.
- (g) The purchaser understands that no representation is made as to the availability of the exemption provided by Rule 144 under the U.S. Securities Act for resales of any Offered Shares, as the case may be.
- (h) The purchaser represents that in the normal course of its business, it invests in or purchases securities similar to the Offered Shares and (a) it has such knowledge and experience in financial and business matters such that it is capable of evaluating the merits and risks of an investment in the Offered Shares; (b) it, and any accounts for which it is acting, are able to bear the economic risk of an investment in the Offered Shares for an indefinite period; and (c) it has concluded on the basis of the information available to it that it is able to bear the risks associated with such an investment.
- (i) The purchaser acknowledges that neither the Company nor any person representing the Company has made any representation to it with respect to the Company or the allocation, offering or sale of Offered Shares other than as set forth in this Offering Memorandum that has been delivered to it, and upon which it is solely relying in making its investment decision with respect to the Offered Shares. The purchaser has held and will hold any offering materials, including this Offering Memorandum, it receives directly or indirectly from the Company in confidence, and it understands that any such information received by it is solely for it and is not to be redistributed or duplicated by it. The purchaser acknowledges that it has read and agreed to the matters stated in the section “*Notice to United States investors*” beginning on page v.
- (j) The purchaser is not an affiliate of the Company or a person acting on behalf of such affiliate, and is not in the business of buying and selling securities or, if it is in such business, it did not acquire the Offered Shares from the Company or an affiliate thereof in the initial distribution of such Shares.
- (k) The purchaser understands that these representations and undertakings are required in connection with United States and other securities laws and that the Company and the Managers and their respective affiliates will rely upon the truth and accuracy of the foregoing acknowledgements, representations and agreements. The purchaser irrevocably authorizes the Company and the Global Coordinator and Bookrunner, acting on behalf of the Managers, to produce this Offering Memorandum to any interested party in any administrative or legal proceedings or official inquiry with respect to the matters covered herein.
- (l) The Company will not recognize any offer, sale, pledge or other transfer of the Offered Shares made other than in compliance with the above stated restrictions.
- (m) The purchaser undertakes to promptly notify the Company and the Global Coordinator and Bookrunner, acting on behalf of the Managers, if, at any time prior to the delivery of the Offered Shares, any of the foregoing ceases to be true.

Neither the Company nor any of the Managers accepts any legal responsibility for any violation by any person, whether or not a prospective investor in the Offered Shares, of any of the foregoing restrictions.

In addition, until the end of the 40th calendar day after commencement of this Offering, any offer or sale of the Offered Shares within the United States by a dealer (whether or not participating in this Offering) may violate the registration requirements of the Securities Act if such offer or sale is made other than in accordance with Rule 144A or another exemption from registration under the Securities Act.

### C. **European economic area**

In relation to each Relevant Member State, with effect from and including the Relevant Implementation Date, an offer to the public of any Shares that are the subject of the Offering contemplated by this Offering Memorandum may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State may be made at any time, with effect from and including the Relevant Implementation Date, under the following exemptions under the Prospectus Directive:

- (a) to any legal entity that is a qualified investor as defined in the Prospectus Directive; or
- (b) in any other circumstances falling within article 3(2) of the Prospectus Directive,

provided that no such offer of shares shall require the Company or the Managers to publish a prospectus pursuant to article 3 of the Prospectus Directive or supplement a prospectus pursuant to article 16 of the Prospectus Directive.

For the purposes of this provision, the expression an “**offer to the public**” in relation to any shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the shares to be offered so as to enable an investor to decide to purchase or subscribe the shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State.

This European Economic Area selling restriction is in addition to any other selling restrictions set out in this Offering Memorandum.

### D. **United Kingdom**

This Offering Memorandum is only directed at, and will only be provided to, persons to whom interests may lawfully be promoted pursuant to section 21 of the Financial Services and Markets Act 2000 (the “**FSMA**”). In particular, this Offering Memorandum is only directed at and will only be provided to investment professionals within the meaning of article 19 of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005 (“**FPO**”) (“**Relevant Persons**”). Any investment or investment activity to which this Offering Memorandum relates is available only to Relevant Persons and will be engaged in only with Relevant Persons. Persons who are not investment professionals within the meaning of article 19 of the FPO should not rely on this Offering Memorandum.

This Offering Memorandum has not been delivered for approval to the Financial Conduct Authority (“**FCA**”) in the United Kingdom or to an authorized person within the meaning of FSMA. No approved prospectus within the meaning of section 85 of FSMA or of the Prospectus Directive has been published or is intended to be published in relation to the Offering. This Offering Memorandum does not constitute a Offering Memorandum for the purposes of FSMA or the Prospectus Directive. As used herein, “**United Kingdom**” means the United Kingdom of Great Britain and Northern Ireland.

### E. **Australia**

This Offering Memorandum and the Offering is only made available in Australia to persons to whom a disclosure document is not required to be given under Chapter 6D of the Corporations Act 2001. This Offering Memorandum is not a prospectus, product disclosure statement or any other form of formal “disclosure document” for the purposes of the Corporations Act, and is not required to, and does not, contain all the information that would be required in a disclosure document under the Corporations Act. If you are

in Australia, this document is made available to you provided you are a person to whom an offer of securities can be made without a disclosure document such as a professional investor or sophisticated investor for the purposes of Chapter 6D of the Corporations Act.

This Offering Memorandum has not been, and will not be, lodged with the Australian Securities and Investments Commission (“**ASIC**”) as a disclosure document for the purpose of the Corporations Act 2001. No Share may be offered for sale (or transferred, assigned or otherwise alienated) to investors in Australia for at least 12 months after this issue, except in circumstances where disclosure to investors is not required under Chapter 6D of the Corporations Act 2001 or unless a disclosure document that complies with the Corporations Act 2001 is lodged with the ASIC. Each investor acknowledges the above and, by applying for securities under this Offering Memorandum, gives an undertaking not to sell those securities (except in the circumstances referred to above) for 12 months after their issue.

The persons referred to in this Offering Memorandum may not hold Australian financial services licenses and may not be licensed to provide financial product advice in relation to the Shares. No “cooling-off” regime will apply to an acquisition of any interest in the Company.

This Offering Memorandum does not take into account the investment objectives, financial situation or needs of any particular person. Accordingly, before making any investment decision in relation to this Offering Memorandum, you should assess whether the acquisition of any interest in the Company is appropriate in light of your own financial circumstances or seek professional advice.

#### **F. Japan**

The Shares have not been and will not be registered under the Financial Instruments and Exchange Law, as amended (the “**FIEL**”). This Offering Memorandum is not an offer of securities for sale, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means any person resident in Japan, including any corporation or entity organized under the laws of Japan) or to others for reoffer or resale, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan, except pursuant to an exemption from the registration requirements under the FIEL and otherwise in compliance with such law and any other applicable laws, regulations and ministerial guidelines of Japan.

#### **G. Canada**

The Offered Shares may not, directly or indirectly, be offered, sold or distributed within Canada, or to, or for the benefit or account of, any resident of Canada, except in compliance with all applicable securities laws, regulations or rules of the provinces and territories of Canada and with the prior approval of the Global Coordinator and Bookrunner, acting on behalf of the Managers. This Offering Memorandum, or any other material relating to the Offered Shares, may not be distributed or delivered in Canada, except in compliance with all applicable securities laws, regulations or rules of the provinces and territories of Canada. No securities commission or similar authority in Canada has reviewed or in any way passed upon this Offering Memorandum or the merits of the Offered Shares, and any representation to the contrary is an offence.

The Company is not a reporting issuer in any province or territory of Canada and all of its executive management and directors are ordinarily resident outside of Canada. The Offered Shares are being sold primarily outside Canada and may be sold in Canada only to purchasers resident or located in the Provinces of Ontario, Quebec, Alberta and British Columbia (the “**Canadian Jurisdictions**”), purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions (“**NI 45-106**”) or the Securities Act (Ontario) (the “**OSA**”), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations (“**NI 31-103**”). Any resale of the Offered Shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable Canadian securities laws and purchasers in the Canadian Jurisdictions should consult with a legal advisor prior to any resale of the Offered Shares whether in Canada or elsewhere.

Each purchaser of Offered Shares in the Canadian Jurisdictions will be deemed to have represented and agreed as follows:

- (a) The purchaser is purchasing, or is deemed to be purchasing, the Offered Shares as principal for investment purposes and not with a view to resale or further distribution.
- (b) The purchaser is not an individual and is resident in one of the Canadian Jurisdictions.
- (c) The purchaser is an accredited investor as defined in NI 45-106 and the OSA (other than a person that was created or is used solely to purchase or hold securities as an accredited investor).
- (d) The purchaser is a permitted client as defined in NI 31-103.
- (e) The purchaser will provide all information and documentation reasonably requested by the Company or the Managers to establish that the purchaser is an accredited investor (and the applicable paragraph number in the definition thereof) and a permitted client, and to permit them to complete any reports required to be filed in any Canadian Jurisdiction.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this Offering Memorandum (including any amendment hereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts ("**NI 33-105**"), the Managers are relying on the exemption therein from the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with the Offering.

In connection with the subscription for the Offered Shares, the purchaser has required that all documents relating thereto be drawn up in the English language only. Dans le cadre de la souscription pour des actions offertes, l'acquéreur a requis que tous les documents s'y rattachant soient rédigés en anglais seulement.

## **XX. TAXATION**

### **A. Swiss taxation**

*The following summary does not purport to address all tax consequences of the Offering, the acquisition, the ownership and sale or other disposition of Shares and does not take into account the specific circumstances of any particular investor. This summary is based on the tax laws, regulations and regulatory practices of Switzerland as in effect on the date hereof, which are subject to change (or subject to changes in interpretation), possibly with retroactive effect.*

*Current and prospective shareholders are advised to consult their own tax advisers in light of their particular circumstances as to the Swiss tax laws, regulations and regulatory practices that could be relevant for them in connection with the Offering, the acquiring, owning and selling or otherwise disposing of Shares and receiving dividends and similar cash or in-kind distributions on Shares (including dividends on liquidation proceeds and stock dividends) or distributions on Shares based upon a capital reduction (*Nennwertrückzahlungen*) or reserves paid out of capital contributions (*Reserven aus Kapitaleinlagen*) and the consequences thereof under the tax laws, regulations and regulatory practices of Switzerland.*

#### **1. Swiss federal, cantonal and communal individual income tax and corporate income tax**

##### **a. Non-Resident Shareholders**

Shareholders who are not resident in Switzerland for tax purposes, and who, during the relevant taxation year, have not engaged in a trade or business carried on through a permanent establishment or fixed place of business situated in Switzerland for tax purposes (all such shareholders, hereinafter, for the purposes of this section “**Non-Resident Shareholders**”), will not be subject to any Swiss federal, cantonal and communal income tax on dividends and similar cash or in-kind distributions on Shares (including liquidation proceeds and stock dividends) (hereinafter, for the purposes of this section “*Swiss taxation*”, “**Dividends**”), distributions based upon a capital reduction on Shares (*Nennwertrückzahlungen*) and distributions paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*), or capital gains realized on the sale or other disposition of Shares (see, “—*Swiss federal withholding tax*” beginning on page 157 for a summary of Swiss federal withholding tax on Dividends).

##### **b. Resident Private Shareholders**

Swiss resident individuals who hold their Shares as private assets (hereinafter, for the purposes of this section “*Swiss taxation*”, referred to as “**Resident Private Shareholders**”) are required to include Dividends, but not distributions based upon a capital reduction (*Nennwertrückzahlungen*) and distributions paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*), in their personal income tax return and are subject to Swiss federal, cantonal and communal income tax on any net taxable income for the relevant taxation period, including the Dividends, but not the distributions based upon a capital reduction (*Nennwertrückzahlungen*) and the distributions paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*). Capital gains resulting from the sale or other disposition of Shares are not subject to Swiss federal, cantonal and communal income tax, and conversely, capital losses are not tax-deductible for Resident Private Shareholders. However, capital gains upon repurchase of own Shares by the Company may be re-characterized into a taxable Dividend, in some cases and subject to certain conditions (in particular, if the Shares are repurchased for subsequent cancellation, if the repurchased Shares exceed 10% of the Company’s share capital (20% in specific circumstances), or if the holding of such shares by the Company exceeds certain time limits). When such a capital gain is re-characterized as a Dividend, the relevant income for tax purposes corresponds to the difference between the repurchase price and the sum of the nominal value of the repurchased Shares and the reserves from capital contributions (*Reserven aus Kapitaleinlagen*) paid back upon the repurchase. See “—*Domestic Commercial Shareholders*” beginning on page 157 for a summary of the taxation treatment applicable to Swiss resident individuals who, for income tax purposes, are classified as “professional securities dealers”.

### **c. Domestic Commercial Shareholders**

Corporate and individual shareholders who are resident in Switzerland for tax purposes, and corporate and individual shareholders who are not resident in Switzerland, and who, in each case, hold their Shares as part of a trade or business carried on in Switzerland, in the case of corporate and individual shareholders not resident in Switzerland, through a permanent establishment or fixed place of business situated, for tax purposes, in Switzerland, are required to recognize Dividends, distributions based upon a capital reduction (*Nennwertrückzahlungen*) and distributions paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*) and capital gains or losses realized on the sale or other disposition of Shares in their income statement for the relevant taxation period and are subject to Swiss federal, cantonal and communal individual or corporate income tax, as the case may be, on any net taxable earnings for such taxation period. The same taxation treatment also applies to Swiss-resident private individuals who, for income tax purposes, are classified as “professional securities dealers” for reasons of, *inter alia*, frequent dealing, or leveraged investments, in shares and other securities (the shareholders referred to in this paragraph, hereinafter for the purposes of this section “*Swiss taxation*”, as “**Domestic Commercial Shareholders**”). Domestic Commercial Shareholders who are corporate taxpayers may be eligible for dividend relief (*Beteiligungsabzug*) in respect of Dividends and distributions based upon a capital reduction (*Nennwertrückzahlungen*) and distributions paid out of reserves from capital contributions (*Reserven aus Kapitaleinlagen*) if the Shares held by them as part of a Swiss business have an aggregate market value of at least CHF 1 million.

## **2. Swiss cantonal and communal private wealth tax and capital tax**

### **a. Non-Resident Shareholders**

Non-Resident Shareholders are not subject to Swiss cantonal and communal private wealth tax or capital tax.

### **b. Resident Private Shareholders and Domestic Commercial Shareholders**

Resident Private Shareholders and Domestic Commercial Shareholders who are individuals are required to report their Shares as part of their private wealth or their Swiss business assets, as the case may be, and will be subject to Swiss cantonal and communal private wealth tax on any net taxable wealth (including Shares), in the case of Domestic Commercial Shareholders to the extent the aggregate taxable wealth is allocable to Switzerland. Domestic Commercial Shareholders who are corporate taxpayers are subject to Swiss cantonal and communal capital tax on taxable capital to the extent such taxable capital is allocable to Switzerland.

## **3. Swiss federal withholding tax**

Dividends that the Company pays on the Shares are subject to Swiss federal withholding tax (*Verrechnungssteuer*) at a rate of 35% on the gross amount of the Dividend. Furthermore, a repurchase of own Shares by the Company as described above may be characterized as taxable Dividend which is subject to Swiss federal withholding tax if certain conditions are met. In the event of a taxable share repurchase, Swiss federal withholding tax at a rate of 35% is imposed on the difference between the repurchase price and the sum of the nominal value of the repurchased Shares and the reserves of capital contributions (*Reserven aus Kapitaleinlagen*) paid back upon the repurchase. The Company is required to withhold the Swiss federal withholding tax from the Dividend and remit it to the Swiss Federal Tax Administration. Distributions based upon a capital reduction (*Nennwertrückzahlungen*) and distributions paid out from reserves of capital contributions (*Reserven aus Kapitaleinlagen*) are not subject to Swiss federal withholding tax.

The Swiss federal withholding tax on a Dividend will be refundable in full to a Resident Private Shareholder and to a Domestic Commercial Shareholder, who, in each case, *inter alia*, as a condition to a refund, duly reports the Dividend in his individual income tax return as income or recognizes the Dividend in his income statement as earnings, as applicable.

A Non-Resident Shareholder may be entitled to a partial refund of the Swiss federal withholding tax on a Dividend if the country of his or her residence for tax purposes has entered into a bilateral treaty for the avoidance of double taxation with Switzerland and the conditions of such treaty are met. Such shareholders should be aware that the procedures for claiming treaty benefits (and the time required for obtaining a refund) might differ from country to country. The applicable refund request form may be filed with the Swiss Federal Tax Administration following receipt of the Dividend, the relevant deduction certificate, and the relevant tax voucher in case the Shares are held in a non-Swiss deposit, however no later than December 31 of the third year following the calendar year in which the Dividend was payable.

#### **4. Swiss federal stamp duties**

The Company is subject to and has to pay to the Swiss Federal Tax Administration a 1% Swiss federal issuance stamp duty (*Emissionsabgabe*) on the consideration received by it for the issuance of the New Shares. The issuance and the delivery of the newly created Offered Shares to the initial shareholders at the Offer Price is not subject to Swiss federal securities turnover stamp duty (*Umsatzabgabe*).

Any subsequent dealings in the Shares where a bank or another securities dealer in Switzerland or Liechtenstein (as defined in the Swiss Federal Stamp Tax Act) acts as an intermediary, or is a party to the transaction, are subject to Swiss federal securities turnover stamp duty (*Umsatzabgabe*) at an aggregate tax rate of up to 0.15% of the consideration paid for such Shares, subject to certain exemptions provided for in the Swiss Federal Stamp Tax Act.

#### **5. Automatic exchange of information**

On November 19, 2014, Switzerland signed the Multilateral Competent Authority Agreement, which is based on article 6 of the OECD/Council of Europe administrative assistance convention and is intended to ensure the uniform implementation of automatic exchange of information (the “**AEOI**”). The Federal Act on the International Automatic Exchange of Information in Tax Matters (the “**AEOI Act**”) entered into force on January 1, 2017. The AEOI Act is the legal basis for the implementation of the AEOI standard in Switzerland.

The AEOI is being introduced in Switzerland through bilateral agreements or multilateral agreements. The agreements have, and will be, concluded on the basis of guaranteed reciprocity, compliance with the principle of specialty (*i.e.*, the information exchanged may only be used to assess and levy taxes (and for criminal tax proceedings)) and adequate data protection.

Based on such multilateral agreements and bilateral agreements and the implementing laws of Switzerland, Switzerland collects and exchanges data in respect of financial assets, including the Shares, held in, and income derived thereon and credited to, accounts or deposits with a paying agent in Switzerland for the benefit of individuals resident in a EU Member State or in a treaty state.

#### **6. Swiss Facilitation of the Implementation of the U.S. Foreign Account Tax Compliance Act**

Switzerland has concluded an intergovernmental agreement with the U.S. to facilitate the implementation of FATCA. The agreement ensures that the accounts held by U.S. persons with Swiss financial institutions are disclosed to the U.S. tax authorities either with the consent of the account holder or by means of group requests within the scope of administrative assistance. Information will not be transferred automatically in the absence of consent, and instead will be exchanged only within the scope of administrative assistance on the basis of the double taxation agreement between the U.S. and Switzerland. On October 8, 2014, the Swiss Federal Council approved a mandate for negotiations with the U.S. on changing the current direct-notification-based regime to a regime where the relevant information is sent to the Swiss Federal Tax Administration, which in turn provides the information to the U.S. tax authorities.

## B. U.S. federal income taxation of Offered Shares

The following is a summary of certain U.S. federal income tax considerations of acquiring, holding and disposing of Offered Shares. This summary is based on the U.S. Internal Revenue Code of 1986 (the “Code”), final, temporary and proposed U.S. Treasury regulations, administrative and judicial interpretations, all as of the date hereof and all of which are subject to change, possibly with retroactive effect, as well as on the income tax treaty between the United States and Switzerland as currently in force (the “Treaty”).

This summary does not discuss all aspects of U.S. federal income taxation that may be relevant to investors, in light of their particular circumstances, such as investors subject to special tax rules (including, without limitation: (i) financial institutions; (ii) insurance companies; (iii) traders or dealers in stocks, securities, or currencies or notional principal contracts; (iv) regulated investment companies; (v) real estate investment trusts; (vi) tax-exempt organizations; (vii) entities that are treated as partnerships, or pass-through entities for U.S. federal income tax purposes, or persons that hold Offered Shares through such entities; (ix) holders that own (directly, indirectly or constructively) 10 per cent. or more of the interests (by vote or value) of the Company treated as equity for U.S. federal income tax purposes; (x) investors that hold Offered Shares as part of a straddle, hedge, conversion, constructive sale or other integrated transaction for U.S. federal income tax purposes; (xi) U.S. Holders (as defined below) that have a functional currency other than the U.S. dollar; (xii) U.S. expatriates and former long-term residents of the United States; (xiii) investors holding the Offered Shares in connection with a trade or business conducted outside of the United States; and (xiv) individual retirement accounts and other tax-deferred accounts), all of whom may be subject to tax rules that differ significantly from those summarized below. This summary does not address tax consequences applicable to holders of equity interests in a holder of the Offered Shares, U.S. federal estate, gift, alternative minimum tax considerations, Medicare contribution tax considerations, or non-U.S., state or local tax considerations. This summary only addresses investors that will acquire Offered Shares in the Offering, and it assumes that investors will hold their Offered Shares as capital assets (generally, property held for investment).

For the purposes of this summary, a “**U.S. Holder**” is a beneficial owner of Offered Shares that is for U.S. federal income tax purposes (i) an individual who is a citizen or resident of the United States, (ii) a corporation created in, or organized under the laws of, the United States or any state thereof, including the District of Columbia, (iii) an estate the income of which is includible in gross income for U.S. federal income tax purposes regardless of its source or (iv) a trust that is subject to U.S. tax on its worldwide income regardless of its source. A “**Non-U.S. Holder**” is a beneficial owner of Offered Shares that is neither a U.S. Holder nor a partnership.

If an entity or arrangement treated as a partnership for U.S. federal income tax purposes holds Offered Shares, the tax treatment of a partner in such partnership generally will depend upon the status of the partner and the activities of the partnership. Any such partner or partnership should consult their tax advisors as to the U.S. federal income tax consequences to them of the acquisition, ownership and disposition of Offered Shares.

THE SUMMARY OF U.S. FEDERAL INCOME TAX CONSEQUENCES SET OUT BELOW IS FOR GENERAL INFORMATION ONLY. ALL PROSPECTIVE PURCHASERS SHOULD CONSULT THEIR TAX ADVISORS AS TO THE PARTICULAR TAX CONSEQUENCES TO THEM OF ACQUIRING, OWNING, AND DISPOSING OF THE OFFERED SHARES, INCLUDING THEIR ELIGIBILITY FOR THE BENEFITS OF THE TREATY, THE APPLICABILITY AND EFFECT OF STATE, LOCAL, NON-U.S. AND OTHER TAX LAWS AND POSSIBLE CHANGES IN TAX LAW.

### 1. Passive foreign investment company rules

If the Company is classified as a passive foreign investment company (a “**PFIC**”) in any taxable year in which a U.S. Holder holds Shares, such U.S. Holder may be subject to significant adverse tax consequences. The Company has not determined whether it was a PFIC for the taxable year ended December 31, 2017,

and, because the factual elements underlying this analysis are subject to change (in particular based on fluctuations in market conditions), and because the interpretation of the law relating to PFIC status is not clear in all respects, the Company cannot provide assurances as to whether it will be classified as a PFIC in the current taxable year or in the future.

The Company will be classified as a PFIC in respect of any taxable year in which, after taking into account its income and gross assets (and the income and assets of certain affiliates pursuant to applicable “look-through rules”) either (i) 75% or more of its gross income consists of certain types of “passive income” or (ii) 50% or more of the average quarterly value of its assets is attributable to “passive assets” (assets that produce or are held for the production of passive income). Passive income generally includes interest, dividends, rents, certain non-active royalties and capital gains. PFIC status is a factual determination that needs to be made annually after the close of each taxable year, on the basis of the composition of the Company’s income and assets, the relative value of its active and passive assets from time to time, and its market capitalization. For this purpose, certain of the Company’s assets are treated as passive even though it holds them in the ordinary course of its business operations. The composition of the Company’s income and assets will be affected by how, and how quickly, the Company uses the proceeds from this Offering. Under circumstances where the cash is not deployed for active purposes, the Company’s risk of becoming a PFIC may increase. Because (i) the Company currently owns, and will own after the completion of the Offering, a substantial amount of passive assets, including cash, and (ii) the values of the Company’s assets, including intangible assets, are uncertain and may vary substantially over time, there is a material risk that the Company will be, and there can be no assurance that the Company will not be, a PFIC in 2018 or any future year.

If the Company is classified as a PFIC in any year that a U.S. Holder is a shareholder, the Company generally will continue to be treated as a PFIC for that U.S. Holder in all succeeding years, regardless of whether the Company continues to meet the income or asset test described above.

If the Company is a PFIC for any taxable year during which a U.S. Holder holds Offered Shares and such U.S. Holder does not make a valid election as discussed below, the U.S. Holder will be subject to special tax rules with respect to any “excess distribution” received and any gain realized from a sale or other disposition (including a pledge) of Offered Shares. Distributions received in a taxable year that are greater than 125 per cent. of the average annual distributions received during the shorter of the three preceding taxable years or the U.S. Holder’s holding period for the Offered Shares will be treated as excess distributions. Under these special tax rules: (i) the excess distribution or gain will be allocated rateably over the U.S. Holder’s holding period for the Offered Shares; (ii) the amount allocated to the current taxable year and other years before the Company was a PFIC will be treated as ordinary income; and (iii) the amount allocated to each other year will be subject to tax at the highest tax rate in effect for that year and an interest charge (at the rate generally applicable to underpayments of tax for the period from such year to the current year) will be imposed on the resulting tax attributable to each such year. Additionally, dividends paid by the Company would not be eligible for the reduced rate of tax described below under “—Distributions” beginning on page 161.

Furthermore, if the Company is a PFIC with respect to a U.S. Holder for any taxable year, to the extent any of the Company’s subsidiaries are also PFICs, the U.S. Holder may be deemed to own shares in such lower-tier PFICs that are directly or indirectly owned by the Company in the proportion to which the value of Offered Shares such U.S. Holder owns bears to the value of all of the Shares, and the U.S. Holder may be subject to the tax consequences described above with respect to the shares of such lower-tier PFIC that such U.S. Holder would be deemed to own. As a result, if the Company is a PFIC and received a distribution from any lower-tier PFIC or if any shares in a lower-tier PFIC are disposed of (or deemed disposed of), a U.S. Holder may be subject to tax under the PFIC rules described above in the same manner as if the U.S. Holder had held its proportionate share of the lower-tier PFIC stock directly even though such U.S. Holder has not received the proceeds of the distribution or disposition directly. U.S. Holders should consult their tax advisors regarding the application of the PFIC rules to any of the Company’s subsidiaries.

A U.S. Holder subject to the PFIC rules discussed above or below is required to file Internal Revenue Service (“IRS”) Form 8621 with respect to its investment in the Offered Shares in the year such U.S. Holder

receives any distribution upon, or makes any disposition of, such shares. Prospective purchasers should consult their tax advisors regarding the requirement to file IRS Form 8621 and the potential application of the PFIC regime.

**a. Mark-to-market election**

To mitigate certain adverse consequences of the PFIC rules discussed above, a U.S. Holder may make an election to include gain or loss on the Offered Shares as ordinary income or loss under a mark-to-market method, provided that the Offered Shares are regularly traded on a qualified exchange. Application has been made to, and approval has been given subject to certain conditions by, the SIX Swiss Exchange to list the Offered Shares on the SIX Swiss Exchange, which the Company expects to be a “qualified exchange” for this purpose. No assurance can be given that the Offered Shares will be “regularly traded” for purposes of the mark-to-market election.

If a U.S. Holder makes an effective mark-to-market election, the U.S. Holder will include in each year as ordinary income the excess of the fair market value of its Offered Shares at the end of the year over its adjusted tax basis in the Offered Shares. The U.S. Holder will be entitled to deduct as an ordinary loss each year the excess of its adjusted tax basis in the Offered Shares over their fair market value at the end of the year, but only to the extent of the net amount of gains previously included in income as a result of the mark-to-market election. If a U.S. Holder makes the election, the U.S. Holder’s adjusted tax basis in the Offered Shares will be increased by the amount of any income inclusion and decreased by the amount of any deductions under the mark-to-market rules. In addition, gains from an actual sale or other disposition of Offered Shares will be treated as ordinary income, and any losses will be treated as ordinary losses to the extent of any mark-to-market gains for prior years.

If a U.S. Holder makes a mark-to-market election, it will be effective for the taxable year for which the election is made and all subsequent taxable years unless the Offered Shares are no longer regularly traded on a qualified exchange or the IRS consents to the revocation of the election.

**b. Qualified electing fund election**

To mitigate certain adverse consequences of the PFIC rules discussed above, a U.S. Holder may make an election to treat the Company as a qualified electing fund (“**QEF**”) for U.S. federal income tax purposes. To make a QEF election, the Company must provide U.S. Holders with information compiled according to U.S. federal income tax principles. The Company currently does not intend to compile such information for U.S. Holders, and therefore it is expected that this election will be unavailable.

**2. Distributions**

Subject to the PFIC rules discussed above, a distribution made by the Company on the Offered Shares, including amounts withheld in respect of any Swiss withholding tax thereon, as discussed above under “—*Swiss federal withholding tax*” beginning on page 157, generally will be treated as a dividend includible in the gross income of a U.S. Holder as ordinary income to the extent of the Company’s current and accumulated earnings and profits as determined under U.S. federal income tax principles. To the extent the amount of such distribution exceeds the Company’s current and accumulated earnings and profits as so computed, the distribution will be treated first as a non-taxable return of capital to the extent of such U.S. Holder’s adjusted tax basis in the Offered Shares and, to the extent the amount of such distribution exceeds such adjusted tax basis, will be treated as gain from the sale of such Offered Shares. The Company does not expect to maintain calculations of earnings and profits for U.S. federal income tax purposes. Therefore, a U.S. Holder should expect that such distribution will generally be reported as a dividend. Such dividends will not be eligible for the dividends received deduction allowed to corporations. U.S. Holders should consult their own tax advisors with respect to the appropriate U.S. federal income tax treatment of any distribution received from the Company.

“Qualified dividend income” received by individual and certain other non-corporate U.S. Holders is currently subject to reduced rates applicable to long-term capital gain if (i) the Company is a “qualified foreign

corporation” (as defined below) and (ii) such dividend is paid on Offered Shares that have been held by such U.S. Holder for at least 61 days during the 121-day period beginning 60 days before the ex-dividend date. The Company generally will be a “qualified foreign corporation” if (1) it is eligible for the benefits of the Treaty and (2) it is not a PFIC in the taxable year of the distribution or the immediately preceding taxable year. No assurance can be given that the Company will be eligible for the benefits of the Treaty. See above under “*Passive foreign investment company rules*” beginning on page 159 for a discussion of the Company’s PFIC status.

Dividends on the Offered Shares generally will constitute income from sources outside the United States for foreign tax credit limitation purposes.

The U.S. dollar value of any distribution made by the Company in currency other than U.S. dollars (for the purposes of this section “—*U.S. federal income taxation of Offered Shares*” only, a “**foreign currency**”) must be calculated by reference to the exchange rate in effect on the date of receipt of such distribution by the U.S. Holder, regardless of whether the foreign currency is in fact converted into U.S. dollars. If the foreign currency so received is converted into U.S. dollars on the date of receipt, such U.S. Holder generally will not recognize foreign currency gain or loss on such conversion. If the foreign currency so received is not converted into U.S. dollars on the date of receipt, such U.S. Holder will have a basis in the foreign currency equal to its U.S. dollar value on the date of receipt. Any gain or loss on a subsequent conversion or other disposition of the foreign currency generally will be treated as ordinary income or loss to such U.S. Holder and generally will be income or loss from sources within the United States for foreign tax credit limitation purposes.

A U.S. Holder generally will be entitled, subject to certain limitations, to a credit against its U.S. federal income tax liability, or a deduction in computing its U.S. federal taxable income, for Swiss taxes withheld by the Company on dividends. Dividends generally will constitute “passive category income” for purposes of the foreign tax credit. The rules governing foreign tax credits are complex. Prospective investors should consult their tax advisors concerning the foreign tax credit implications of Swiss withholding taxes.

### **3. Sale or other disposition**

Subject to the PFIC rules discussed above, a U.S. Holder generally will recognize gain or loss for U.S. federal income tax purposes upon a sale or other disposition of its Offered Shares in an amount equal to the difference between the amount realized from such sale or disposition and the U.S. Holder’s adjusted tax basis in such Offered Shares, in each case as determined in U.S. dollars. U.S. Holders should consult their tax advisors about how to account for proceeds received on the sale or other disposition of Offered Shares that are not paid in U.S. dollars.

Any such gain or loss generally will be capital gain or loss and will be long-term capital gain (taxable at a reduced rate for non-corporate U.S. Holders, such as individuals) or loss if, on the date of sale or disposition, such Offered Shares were held by such U.S. Holder for more than one year. The deductibility of capital loss is subject to significant limitations. Such gain or loss realized generally will be treated as derived from U.S. sources.

### **4. Non-U.S. Holders**

Subject to the below discussion of backup withholding tax, a non-U.S. Holder generally should not be subject to U.S. federal income or withholding tax on any distributions made on the Offered Shares or gain from the sale, redemption or other disposition of the Securities unless: (i) that distribution and/or gain is effectively connected with the conduct by that non-U.S. Holder of a trade or business in the United States; or (ii) in the case of any gain realized on the sale or exchange of Offered Shares by an individual non-U.S. Holder, that non-U.S. Holder is present in the United States for 183 days or more in the taxable year of the sale, exchange or retirement and certain other conditions are met.

## **5. U.S. information reporting and backup withholding tax**

Payments made through a U.S. paying agent or U.S. intermediary to a U.S. Holder may be subject to information reporting unless the U.S. Holder establishes that payments to it are exempt from these rules. Payments that are subject to information reporting may be subject to backup withholding if a U.S. Holder does not provide its taxpayer identification number and otherwise comply with the backup withholding rules. Non-U.S. Holders may be required to comply with applicable certification procedures to establish that they are not U.S. Holders in order to avoid the application of such information reporting requirements and backup withholding.

Backup withholding is not an additional tax. Amounts withheld under the backup withholding rules are available to be credited against a U.S. Holder's U.S. federal income tax liability and may be refunded to the extent they exceed such liability, provided the required information is timely provided to the IRS.

Certain U.S. Holders that own "specified foreign financial assets" that meet certain U.S. dollar value thresholds generally are required to file an information report with respect to such assets with their tax returns. The Offered Shares generally will constitute specified foreign financial assets subject to these reporting requirements unless the Offered Shares are held in an account at certain financial institutions. U.S. Holders are urged to consult their tax advisors regarding the application of these disclosure requirements to their ownership of the Offered Shares.

### **C. Taxes levied by other jurisdictions**

Jurisdictions outside Switzerland typically also levy taxes in connection with the ownership, or transactions involving, shares or dividend payments or other proceeds derived therefrom.

## **XXI. GENERAL INFORMATION**

### **A. Legal form, jurisdiction, incorporation, name, register, duration, registered address, head office**

The Company, Santhera Pharmaceuticals Holding AG, is a stock corporation (*Aktiengesellschaft*) organized under the laws of Switzerland in accordance with articles 620 et seq. CO and has its seat in Pratteln, Canton of Basel-Landschaft. The Company was incorporated on July 16, 2002, and is registered in the commercial register of the Canton of Basel-Landschaft (register number CHE-105.388.338). The duration of the Company is unlimited. The Articles in effect at the date of this Offering Memorandum are dated November 21, 2018.

The Company's registered address (*Domiziladresse*) is at Hohenrainstrasse 24, 4133 Pratteln, Switzerland.

### **B. Purpose and financial year**

The Company is a holding company and does not carry out operative activities. The Company's business purpose according to the Articles is to acquire, hold, permanently manage, dispose of and finance participations in and outside of Switzerland. The Company may establish branches in and outside of Switzerland, may provide security for other group companies and enter into guaranty obligations, and may conduct any business that seems appropriate to foster or is related to its purpose. The Company may acquire, manage, exploit commercially and sell real estate and intellectual property in and outside of Switzerland and finance other companies.

Pursuant to the Articles, the Company's financial year is determined by the Board. Currently, the Company's financial year corresponds to the calendar year.

### **C. Auditors**

The Company's auditors are appointed on an annual basis. The current auditors are Ernst & Young AG, Aeschengraben 9, 4051 Basel, Switzerland, who have acted as the Company's auditors for more than a decade.

The Audited Consolidated Financial Statements and the Statutory Financial Statements, which are included elsewhere in this Offering Memorandum, have been audited by EY, as stated in their respective reports appearing therein.

According to the Articles, the auditors are elected (or re-elected, as the case may be) at each annual general meeting of shareholders for a term of office until the completion of the following annual general meeting.

### **D. Notices**

In accordance with the Articles, unless personal notification is mandated by law, notices to shareholders are validly made by publication in the Swiss Official Gazette of Commerce (*Schweizerisches Handelsamtsblatt*). Written communications by the Company to its shareholders may be sent by ordinary mail to the last address of the relevant shareholder recorded in the Share Register. Announcements concerning information with significant relevance for the price of the Shares or the Bonds will be made as required and by the means provided by the SIX Swiss Exchange ad-hoc publicity regulations.

### **E. Information policy**

The Company releases its annual financial results in the form of an annual report. Its annual report is published in print and electronic form within four months after the December 31 balance sheet date. In addition, results for the first half of each financial year are released in electronic form within three months after the

June 30 balance sheet date. The Company's annual report and half-year results are currently announced via press releases, press conferences and investor conference calls.

### ***Weblinks***

The Company's website is <http://www.santhera.com>. Information on the Company's website is not part of or incorporated by reference into this Offering Memorandum.

Email distribution list (push system):

<http://www.santhera.com/investors-and-media/news-and-media-center/news-subscriptions>.

Ad hoc messages (pull system):

<http://www.santhera.com/investors-and-media/news-and-media-center/press-releases>.

### **F. Security numbers and ticker symbol**

The SIX Swiss Exchange Ticker Symbol, Swiss Security Number and International Security Identification Number of the Shares are as follows:

SIX Swiss Exchange Ticker Symbol	SANN
Swiss Security Number ( <i>Valorenummer</i> )	2'714'864
International Security Identification Number (ISIN)	CH 0027148649

### **G. Paying agent**

The Company's principal paying agent (*Hauptzahlstelle*) in Switzerland is Basellandschaftliche Kantonalbank.

### **H. Listing agent**

Homburger AG, as recognized representative according to article 43 of the Listing Rules, has filed the application for the listing of the Shares on the SIX Swiss Exchange according to the International Reporting Standard on behalf of the Company.

### **I. Listing and trading**

Application has been made and approval has been given to list the New Shares on the SIX Swiss Exchange according to the International Reporting Standard. The Idorsia Shares were listed on the SIX Swiss Exchange according to the International Reporting Standard on November 22, 2018. The Company expects that the Offered Shares will be listed on the SIX Swiss Exchange according to the International Reporting Standard on or around December 18, 2018 (*i.e.*, the First Day of Trading).

### **J. Notification/amendments or changes**

Any notices containing or announcing amendments to this Offering Memorandum will be announced through electronic media. Notices required under the Listing Rules will be published in electronic form on the website of the SIX Swiss Exchange (currently: [https://www.six-group.com/exchanges/news/official\\_notices/search\\_en.html](https://www.six-group.com/exchanges/news/official_notices/search_en.html)). Changes so notified will be deemed to constitute an amendment or supplement of this Offering Memorandum.

**K. No material changes**

Except as disclosed in this Offering Memorandum, no material changes have occurred in the Company's assets and liabilities, financial position or profits and losses since September 30, 2018.

**L. Applicable law and jurisdiction**

This Offering Memorandum is governed by Swiss law. Any disputes arising under or in connection with this Offering Memorandum shall be settled by the competent courts in Zurich, Canton of Zurich, Switzerland.

## INDEX TO FINANCIAL STATEMENTS

### Excerpts from Santhera Q3 Report 2018

Unaudited interim condensed consolidated financial statements .....	F-2
Unaudited interim statutory financial statements of the Company .....	F-17

### Excerpts from Santhera Annual Report 2017

Consolidated financial statements .....	F-26
Report of the statutory auditor on the consolidated financial statements .....	F-70
Statutory financial statements of the Company .....	F-74
Report of the statutory auditor on the statutory financial statements .....	F-86

### Excerpts from Santhera Annual Report 2016

Consolidated financial statements .....	F-89
Report of the statutory auditor on the consolidated financial statements .....	F-132
Statutory financial statements of the Company .....	F-135
Report of the statutory auditor on the statutory financial statements .....	F-145

### Excerpts from Santhera Annual Report 2015

Consolidated financial statements .....	F-147
Report of the statutory auditor on the consolidated financial statements .....	F-187
Statutory financial statements of the Company .....	F-189
Report of the statutory auditor on the statutory financial statements .....	F-199

# Interim Condensed Consolidated Financial Statements

## Contents

Interim Consolidated Balance Sheet .....	3
Interim Consolidated Income Statement (Unaudited) .....	4
Interim Consolidated Statement of Comprehensive Income (Unaudited).....	5
Interim Consolidated Statement of Cash Flows (Unaudited) .....	6
Interim Consolidated Statement of Changes in Equity (Unaudited) .....	7
Notes to the Interim Condensed Consolidated Financial Statements (Unaudited) .....	8
1 General Information .....	8
2 Summary of Significant Accounting Policies .....	8
3 Seasonality .....	9
4 Exchange Rates of Principal Currencies .....	10
5 Inventories .....	10
6 Cash and Cash Equivalents and Restricted Cash .....	10
7 Share Capital .....	11
8 Transaction with Polyphor .....	11
9 Financial Assets and Liabilities .....	12
10 Segment and Geographic Information.....	13
11 Operating Expenses by Nature.....	14
12 Income taxes .....	14
13 Equity Rights Plans .....	14
14 Related Party Transactions .....	16
15 Subsequent Events.....	16

## Interim Consolidated Balance Sheet

	in CHF thousands	Notes	Sep. 30, 2018 (unaudited)	Dec. 31, 2017 (audited)
<b>Assets</b>				
Tangible assets		10	2,344	2,157
Intangible assets		8, 10	27,443	23,560
Financial assets long-term			775	713
Deferred tax assets			1,245	1,242
Restricted cash long-term		6	1,500	4,500
<b>Noncurrent assets</b>			<b>33,307</b>	<b>32,172</b>
Prepaid expenses and accrued income			804	853
Inventories		5	9,319	10,147
Trade and other receivables			6,953	5,402
Financial assets short-term		9	5,735	13,011
Restricted cash short-term		6	3,000	3,000
Cash and cash equivalents		6	19,654	45,195
<b>Current assets</b>			<b>45,465</b>	<b>77,608</b>
<b>Total assets</b>			<b>78,772</b>	<b>109,780</b>
<b>Equity and liabilities</b>				
Share capital		7	6,528	6,289
Capital reserves and share premium			403,159	392,002
Retained earnings			-400,004	-360,081
Employee benefit reserve			-2,969	-4,905
Treasury shares			-1,145	-335
Other components of equity			-766	-714
<b>Total equity</b>			<b>4,803</b>	<b>32,256</b>
Senior unsecured convertible bonds		9	54,193	53,111
Derivative financial instruments		9	1,069	2,792
Pension liabilities			6,879	8,375
<b>Total noncurrent liabilities</b>			<b>62,141</b>	<b>64,278</b>
Trade and other payables			3,463	4,734
Accrued expenses			8,365	8,512
<b>Total current liabilities</b>			<b>11,828</b>	<b>13,246</b>
<b>Total liabilities</b>			<b>73,969</b>	<b>77,524</b>
<b>Total equity and liabilities</b>			<b>78,772</b>	<b>109,780</b>

## Interim Consolidated Income Statement (Unaudited)

For the three and nine months ended September 30, in CHF thousands	Notes	Three months, ended Sep. 30, 2018	Three months, ended Sep. 30, 2017	Nine months, ended Sep. 30, 2018	Nine months, ended Sep. 30, 2017
<b>Net sales</b>	<b>10</b>	<b>7,607</b>	<b>5,488</b>	<b>23,634</b>	<b>16,347</b>
Cost of goods sold		-1,165	-1,074	-3,606	-3,028
<i>Of which amortization intangible asset</i>		-760	-760	-2,279	-2,279
Other operating income		1	8	1	243
Development	11	-8,244	-6,465	-27,098	-18,168
Marketing and sales	11	-5,716	-7,287	-18,637	-19,909
General and administrative	11	-3,241	-3,460	-11,292	-9,573
Other operating expenses	11	-112	0	-169	-68
<b>Operating expenses</b>	<b>11</b>	<b>-17,313</b>	<b>-17,212</b>	<b>-57,196</b>	<b>-47,718</b>
<b>Operating result</b>		<b>-10,870</b>	<b>-12,790</b>	<b>-37,167</b>	<b>-34,156</b>
Financial income		344	2,687	2,856	3,533
Financial expenses		-1,759	-1,470	-5,232	-3,605
<b>Result before taxes</b>		<b>-12,285</b>	<b>-11,573</b>	<b>-39,543</b>	<b>-34,228</b>
Income taxes	12	-287	952	-380	895
<b>Net result</b>		<b>-12,572</b>	<b>-10,621</b>	<b>-39,923</b>	<b>-33,333</b>
Basic and diluted loss per share (in CHF)		-1.94	-1.69	-6.19	-5.32

**Interim Consolidated Statement of Comprehensive Income (Unaudited)**

For the three and nine months ended September 30, in CHF thousands	Three months, ended Sep. 30, 2018	Three months, ended Sep. 30, 2017	Nine months, ended Sep. 30, 2018	Nine months, ended Sep. 30, 2017
<b>Net result</b>	<b>-12,572</b>	<b>-10,621</b>	<b>-39,923</b>	<b>-33,333</b>
<i>Items never to be reclassified subsequently to net income in subsequent periods:</i>				
Net actuarial gains/(losses) from defined benefit plans	781	4	1,936	137
<i>Items to be reclassified subsequently to net income in subsequent periods:</i>				
Currency translation differences	-41	57	-52	60
<b>Other comprehensive result</b>	<b>740</b>	<b>61</b>	<b>1,884</b>	<b>197</b>
<b>Total comprehensive result</b>	<b>-11,832</b>	<b>-10,560</b>	<b>-38,039</b>	<b>-33,136</b>

## Interim Consolidated Statement of Cash Flows (Unaudited)

for the nine months ended September 30, in CHF thousands	Notes	2018	2017
<b>Result before taxes</b>		<b>-39,543</b>	<b>-34,228</b>
Depreciation of tangible assets		455	175
Amortization of intangible assets		2,360	2,338
Expenses for equity rights plans		5,241	5,518
Change in fair value of derivatives	9	-1,723	-2,344
Change in fair value of financial assets short-term	9	249	-117
Other non-cash items (Polyphor clinical material)	8	290	0
Change in pension liabilities		440	584
Taxes paid		-383	-293
Change in net working capital		311	-2,046
Total financial result		2,376	71
Interest received		1	0
Interest paid		-3,033	-1,541
<b>Cash flow from operating activities</b>		<b>-32,959</b>	<b>-31,883</b>
Investments in tangible assets		-1,271	-439
Investments in intangible assets		-33	-104
Investments in other financial assets short-term		0	-12,915
Disposal of other financial assets short-term		7,027	0
Investments in other financial assets long-term		-70	-426
Change in restricted cash		3,000	-7,500
<b>Cash flow from investing activities</b>		<b>8,653</b>	<b>-21,384</b>
Proceeds from options exercised		0	21
Proceeds from sale of treasury shares		1,894	7,437
Purchase of treasury shares		-3,049	-7,626
Proceeds from convertible bonds		0	57,269
<b>Cash flow from financing activities</b>		<b>-1,155</b>	<b>57,101</b>
Effects of exchange rate changes on cash and cash equivalents		-80	94
<b>Net increase/(decrease) in cash and cash equivalents</b>		<b>-25,541</b>	<b>3,928</b>
Cash and cash equivalents at January 1		45,195	49,815
<b>Cash and cash equivalents at September 30</b>		<b>19,654</b>	<b>53,743</b>

For a disclosure of the non-cash transaction with Polyphor and the related acquisition of intangible assets and clinical material, see note 8 "Transaction with Polyphor".

## Interim Consolidated Statement of Changes in Equity (Unaudited)

	in CHF thousands	Notes	Share capital	Capital reserves and share premium	Retained earnings	Employee benefit reserve	Treasury shares	Translation differences	Total
<b>Balance at January 1, 2017</b>			<b>6,280</b>	<b>382,322</b>	<b>-308,549</b>	<b>-4,734</b>	<b>-172</b>	<b>-796</b>	<b>74,351</b>
Net result			0	0	-33,333	0	0	0	-33,333
Other comprehensive income			0	0	0	137	0	60	197
<b>Total comprehensive result for the period</b>			<b>0</b>	<b>0</b>	<b>-33,333</b>	<b>137</b>	<b>0</b>	<b>60</b>	<b>-33,136</b>
Share-based payment transactions		11	0	5,518	0	0	0	0	5,518
Capital increase from options exercise			5	16					21
Change in treasury shares			0	11	0	0	-200	0	-189
<b>Balance at September 30, 2017</b>			<b>6,285</b>	<b>387,867</b>	<b>-341,882</b>	<b>-4,597</b>	<b>-372</b>	<b>-736</b>	<b>46,565</b>
<b>Balance at January 1, 2018</b>			<b>6,289</b>	<b>392,002</b>	<b>-360,081</b>	<b>-4,905</b>	<b>-335</b>	<b>-714</b>	<b>32,256</b>
Net result			0	0	-39,923	0	0	0	-39,923
Other comprehensive income			0	0	0	1,936	0	-52	1,884
<b>Total comprehensive result for the period</b>			<b>0</b>	<b>0</b>	<b>-39,923</b>	<b>1,936</b>	<b>0</b>	<b>-52</b>	<b>-38,039</b>
Share-based payment transactions		11	0	5,241	0	0	0	0	5,241
Capital increase Polyphor		8	239	6,261	0	0	0	0	6,500
Change in treasury shares			0	-345	0	0	-810	0	-1,155
<b>Balance at September 30, 2018</b>			<b>6,528</b>	<b>403,159</b>	<b>-400,004</b>	<b>-2,969</b>	<b>-1,145</b>	<b>-766</b>	<b>4,803</b>

## Notes to the Interim Condensed Consolidated Financial Statements (Unaudited)

### 1 General Information

Santhera Pharmaceuticals Holding AG (the **Company**, together with its subsidiaries **Santhera** or **Group**) is a Swiss specialty pharmaceutical company focused on the development and commercialization of products for the treatment of neuro-ophthalmological, neuromuscular and pulmonary diseases, areas which include many orphan and rare indications with high unmet medical needs.

The Company, having the listing of its registered shares (**Shares**) on the SIX Swiss Exchange (**SIX**), is a Swiss stock corporation and the parent company of the Group. Its purpose is to acquire, dispose and manage investments. The Company has its registered offices at Hohenrainstrasse 24 in 4133 Pratteln, Switzerland.

The consolidated interim financial statements were approved for publication by the Board of Directors (**Board**) on November 29, 2018.

### 2 Summary of Significant Accounting Policies

The accounting policies used in the preparation of the interim financial statements are consistent with those used in the preparation of the Group's annual financial statements for the year ended December 31, 2017, except for the adoption of new standards and interpretations as of January 1, 2018, as noted below.

#### ***Basis of preparation***

These unaudited consolidated interim financial statements were prepared in accordance with IAS 34, Interim Financial Reporting, of the International Financial Reporting Standards (**IFRS**) and should be read in conjunction with the annual financial statements for the year ended December 31, 2017.

The presentation currency is Swiss francs (**CHF**). All figures included are rounded to the nearest CHF 1,000 except where otherwise indicated.

#### ***Material uncertainties and going concern***

Santhera is subject to different risks and uncertainties, including but not limited to the uncertainty of the development of its clinical studies, regulatory approval and marketing activities in order to achieve profitability. The Group's ability to continue operations as planned for the next 12 months depends on cash flows from ongoing product sales, the results of its development activities and the capability to raise additional funds through an ordinary capital increase.

Santhera continues to generate increasing income from product sales for the indication Leber's hereditary optic neuropathy (**LHON**). Moreover, the Company has collected additional data in patients with Duchenne muscular dystrophy (**DMD**) needed for submission of a Marketing Authorization Application for this indication in the European Union (**EU**) in early 2019. Santhera also prepares for submission of a New Drug Application for patients with DMD in the United States. Lastly, Santhera entered into an option for a sub-licensing agreement with Idorsia for the steroid vamorolone (see note 15 "*Subsequent Events*" for further discussion), which will require an upfront cash payment of USD 20 million within forty days after November 20, 2018.

Santhera's cash, cash equivalents and short-term financial assets amounted to CHF 25.4 million as of September 30, 2018. A material uncertainty remains as to whether Santhera's funding is sufficient to support its going concern for another twelve months.

In order to effect the upfront cash payment to Idorsia for the rights to vamorolone and to fund its ongoing activities, Santhera's Board of Directors has called an Extraordinary General Meeting (**EGM**) to be held on December 11, 2018, and is proposing to the EGM an ordinary capital increase of up to 3,500,000 registered Shares of the Company with a nominal value of CHF 1 each. Santhera plans to raise approximately CHF 50 million of gross proceeds (see note 15 "*Subsequent Events*" for further discussion). Shareholders should note that whilst the Board and Executive Management continue to apply best efforts to raise additional funds, there is no guarantee that such funds can be raised. The availability of sufficient funds is crucial for Santhera and its ability to continue and grow its operations, including the financing of the upfront cash payment to Idorsia. Based on the Board's and the Executive Management's plan as discussed above, the Board of Directors is confident to ensure business continuation and meet its obligations for a further twelve months. Hence, the interim consolidated financial statements have been prepared on a going concern basis.

### ***Changes in accounting policies***

The accounting policies adopted in the preparation of the interim consolidated financial statements are consistent with those followed in the preparation of the Group's annual consolidated financial statements for the year ended December 31, 2017, except for the adoption of new standards effective as of January 1, 2018. The Group has not early adopted any other standard, interpretation or amendment that has been issued but is not yet effective.

The Group applies, for the first time, IFRS 15 Revenue from Contracts with Customers and IFRS 9 Financial Instruments. Several other amendments and interpretations apply for the first time in 2018, but do not have an impact on the interim consolidated financial statements of Santhera.

IFRS 15 supersedes IAS 11 Construction Contracts, IAS 18 Revenue and related interpretations and it applies to all revenue arising from contracts with customers, unless those contracts are in the scope of other standards. Under IFRS 15, revenue is recognized at an amount that reflects the consideration to which an entity expects to be entitled in exchange for transferring goods or services to a customer. The Group adopted IFRS 15 applying the modified retrospective approach. Revenue from sales of products is recognized at the point in time when the customer obtains control of the goods or services, which is generally upon delivery at the customer. The adoption of IFRS 15 had no material impact on the Group's revenue and profit or loss.

IFRS 9 Financial Instruments replaces IAS 39 Financial Instruments: Recognition and Measurement for annual periods beginning on or after January 1, 2018, bringing together all three aspects of the accounting for financial instruments: classification and measurement, impairment and hedge accounting. The application of the classification and measurement requirements of IFRS 9 had no material impact on the Group's equity and profit or loss. Furthermore, the Group does not apply hedge accounting. IFRS 9 requires Santhera to record expected credit losses (**ECL**) on all of its trade receivables, either on a 12-month or lifetime basis. The Group applied the simplified approach and records lifetime expected losses. Based on the nature of its receivables, the application of the impairment model under IFRS 9 had no material impact on the Group's balance sheet or equity. Consequently, no impact in equity was recorded.

## **3 Seasonality**

The operating result is not subject to significant seasonal variations during the financial year.

## 4 Exchange Rates of Principal Currencies

	Income statement in CHF		Balance sheet in CHF	
	average rates for nine months ended		as of period end	
	Sep. 30, 2018	Sep. 30, 2017	Sep. 30, 2018	Dec. 31, 2017
1 Euro (EUR)	1.1610	1.0943	1.1349	1.1691
1 US dollar (USD)	0.9720	0.9840	0.9773	0.9753
1 British pound (GBP)	1.3134	1.2542	1.2775	1.3173
1 Canadian dollar (CAD)	0.7549	0.7531	0.7509	0.7777

## 5 Inventories

This position consists mainly of active pharmaceutical ingredients and semi-finished products which are kept by Santhera as stock for market supply, development and inventory risk management purposes (security stock) for Raxone.

## 6 Cash and Cash Equivalents and Restricted Cash

### 6.1 Cash and cash equivalents

	in CHF thousands	Sep. 30, 2018	Dec. 31, 2017
Cash at banks and on hand			
in CHF		8,902	34,730
in EUR		7,801	8,152
in USD		1,678	1,496
in GBP		1,033	697
in CAD		152	120
other currencies		88	0
<b>Total at period end</b>		<b>19,654</b>	<b>45,195</b>

### 6.2 Restricted cash

	in CHF thousands	Sep. 30, 2018	Dec. 31, 2017
Long-term		1,500	4,500
Short-term		3,000	3,000
<b>Total at period end</b>		<b>4,500</b>	<b>7,500</b>

Restricted cash is designated for interest payments due related to the convertible bonds during the first 3 years (starting 2017). These funds are kept in an escrow account with the bond agent.

## 7 Share Capital

### 7.1 Ordinary share capital

During the reporting period ending September 30, 2018, 238,924 Shares were issued out of the authorized share capital. With these Shares Santhera obtained from Polyphor, Allschwil, Switzerland, the worldwide, exclusive rights to develop and commercialize POL6014, an innovative macrocycle elastase inhibitor (see note 8 *“Transaction with Polyphor”*).

As a result, as of September 30, 2018, the issued nominal share capital amounted to CHF 6,527,479, divided into 6,527,479 Shares at a nominal value of CHF 1 each.

### 7.2 Authorized share capital

In February 2018, 238,924 Shares were issued out of the authorized share capital in connection with the agreement with Polyphor (see note 8 *“Transaction with Polyphor”*). On the occasion of the Annual General Meeting (AGM) on April 12, 2018, Santhera’s shareholders approved the increase of the authorized share capital of the Company.

The Board is authorized to increase the share capital at any time until April 11, 2020, through the issuance of up to 1,500,000 Shares with a nominal value of CHF 1 each (see note 15 *“Subsequent Events”*).

### 7.3 Conditional share capital

As of September 30, 2018, the Company had conditional share capital, pursuant to which the share capital may be increased by

- (i) a maximum amount of CHF 691,302 (2017: CHF 700,000) through the issuance of up to 691,302 (2017: 700,000) Shares, under the exclusion of shareholders’ pre-emptive rights, for equity rights being exercised under the Company’s equity rights plans (see note 13 *“Equity Rights Plans”*).
- (ii) a maximum amount of CHF 930,000 (2017: CHF 930,000) by issuing up to 930,000 (2017: 930,000) Shares through the exercise of warrants/options and/or notes granted in connection with bonds or similar debt instruments linked with option and/or conversion rights granted by the Company.

## 8 Transaction with Polyphor

On February 15, 2018, Santhera announced that it had entered into a license agreement with Polyphor Ltd., Allschwil, Switzerland, for POL6014, a clinical stage selective inhibitor of human neutrophil elastase with the potential to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases. Under the terms of the agreement, Santhera may be required to make cash payments due to future development, regulatory and sales milestones of up to CHF 121 million (i.e. contingent payments). Consistent with existing licensing agreements, such contingent payments have not been capitalized.

### Significant non-cash transaction

The consideration for the acquisition of the license and the clinical material was paid by issuing shares of Santhera Pharmaceuticals Holding AG for a total amount of CHF 6.5 million (CHF 27.2053 per share; see note 7 *“Share Capital”*). Santhera acquired on one hand a license (POL6014) in the amount of CHF 6.2 million, which was recognized as an addition to the intangible assets. The intangible asset is being developed and hence not yet available for use and not amortized. On the other hand, the Group purchased clinical material in the amount of CHF 0.3 million, which was booked as a development expense. The amounts of the two parts were based on their relative fair values.

## 9 Financial Assets and Liabilities

### 9.1 Financial assets short-term

Financial assets (units in a fund) are measured at fair value through profit or loss and are based on quoted prices (Level 1). A loss of TCHF 254 (financial expenses) resulted during the reporting period (2017: gain of TCHF 117).

### 9.2 Financial liabilities

On February 17, 2017, Santhera issued senior unsecured convertible bonds in the nominal amount of CHF 60 million. The bonds, listed on the SIX, are interest bearing (5%) with a maximum term of 5 years and are convertible into registered Shares of Santhera with a nominal value of CHF 1 each. The initial conversion price was fixed at CHF 86.4006 and has been reset in accordance with the terms of the bond in February 2018 to CHF 64.80. In addition, Santhera may call the convertible bonds at any time on or after the second anniversary of the issue date at par, plus accrued interest, if any, if the VWAP of the Shares is at least 160% of the conversion price. The convertible bonds are measured at amortized costs applying the effective interest method. The fair value of the bonds (Level 1) at September 30, 2018, amounts to CHF 46.7 million (December 31, 2017: CHF 51.6 million).

The embedded financial derivatives (conversion right, reset mechanism and early redemption option) are valued by an independent consultant initially and at period end at fair value, applying a simulation-based valuation approach. The valuation of the embedded derivatives is based on input parameters, classified as Level 3. The simulation is mainly based on the historical volatility of Santhera shares. The period of volatility data used is measured according to the remaining life of the convertible bonds. The volatility used as per September 30, 2018, was at 66.7% (September 30, 2017: 89.3%).

The embedded conversion right and the reset mechanism are directly related and have the same risk exposure. Therefore, these two derivatives are accounted for as a single instrument (i.e. a compound derivative). Due to the reset mechanism, the compound derivative is not settled for a fixed number of equity and hence classifies as a financial liability.

The value of the derivatives initially amounted to CHF 5.3 million (February 17, 2017). At December 31, 2017, the value was CHF 2.8 million and at period end CHF 1.1 million (September 30, 2018). The change in the fair value (CHF 1.7 million) was recognized in financial income.

Sensitivity analysis:

	September 30, 2018		September 30, 2017	
	Increase/decrease in volatility assumption	Effect on result before taxes in CHF thousands	Increase/decrease in volatility assumption	Effect on result before taxes in CHF thousands
Change in volatility	+5%	-111	+5%	163
	-5%	141	-5%	-112

	In CHF thousands	Convertible bonds	Derivative financial instruments
<b>January 1, 2017</b>		<b>0</b>	<b>0</b>
Proceeds from convertible bonds		60,000	0
Transaction costs relating to convertible bonds		-2,731	0
<b>Cash flows in 2017</b>		<b>57,269</b>	<b>0</b>
Non-cash changes			
Initial recognition derivative financial instruments		-5,332	5,332
Change in fair value of derivative financial instruments		0	-2,344
Effective interest/amortized cost calculation		827	0
<b>September 30, 2017</b>		<b>52,764</b>	<b>2,988</b>
Change in fair value of derivative financial instruments		0	-196
Effective interest/amortized cost calculation		347	0
<b>December 31, 2017</b>		<b>53,111</b>	<b>2,792</b>
Change in fair value of derivative financial instruments		0	-1,723
Effective interest/amortized cost calculation		1,082	0
<b>September 30, 2018</b>		<b>54,193</b>	<b>1,069</b>

## 10 Segment and Geographic Information

### 10.1 Segment information

Santhera operates in one business segment, namely development and commercialization of products for the treatment of neuro-ophthalmological, neuromuscular and pulmonary diseases. The Board, the Executive Management and senior managers, being the chief operating decision makers, assess the reporting data and allocate resources as one segment on an aggregated consolidated level according to operating expenses by function. Santhera generates revenue from sales of Raxone for the treatment of LHON. Geographic revenue information is based on location of the customer.

### 10.2 Geographic information

#### Net sales

	nine months ended September 30, in CHF thousands	2018	2017
Europe		23,547	16,282
Rest of the world		87	65
<b>Total</b>		<b>23,634</b>	<b>16,347</b>

In the reporting period 2018, net sales amounted to CHF 23.6 million. Raxone was sold in more than 20 European countries, with the majority of sales generated in France and Germany (in the reporting period 2017, sales went into 18 European countries, with a majority of sales in France and Germany).

**Noncurrent assets (excluding financial instruments and deferred tax assets)**

	in CHF thousands	<b>Sep. 30, 2018</b>	Dec. 31, 2017
Switzerland		29,577	25,451
Rest of Europe		136	171
North America		74	95
<b>Total</b>		<b>29,787</b>	<b>25,717</b>

**11 Operating Expenses by Nature**

	nine months ended September 30, in CHF thousands	<b>2018</b>	<b>2017</b>
External development expenses		-17,248	-10,678
Patent and license expenses		-329	-204
Marketing expenses		-7,538	-9,115
Employee expenses		-26,459	-23,834
<i>Of which non-cash-relevant expenses for share-based payments</i>		-5,241	-5,518
General and administrative expenses		-3,920	-3,074
Depreciation and amortization		-536	-234
Lease expenses		-997	-511
Other operating expenses		-169	-68
<b>Total operating expenses</b>		<b>-57,196</b>	<b>-47,718</b>

Increased expenses for the reporting period in 2018 mainly resulted from additional development activities (e.g. DMD and POL6014) and staff hired for development activities.

**12 Income Taxes**

	nine months ended September 30, in CHF thousands	<b>2018</b>	<b>2017</b>
Current income taxes		-382	-293
Deferred taxes		2	1,188
<b>Total</b>		<b>-380</b>	<b>895</b>

Movements on deferred taxes relate to temporary differences on inventory.

**13 Equity Rights Plans**

Santhera has established equity rights plans to align the long-term interests of the members of the Board, the Executive Management and employees. Rights granted under these plans are equity-settled. New grants are only possible under Share Appreciation Rights Plans (**SARP**).

The fair value of equity rights (share appreciation rights (**SAR**) or stock options) is determined at each grant date by using the Hull-White pricing model. For the calculation of the fair value of SAR granted during the reporting period in 2018 the same range of valuation parameters as disclosed in the financial statements as of December 31, 2017, was applied, except for the exercise prices (equal to the Share prices at grant) which were between CHF 16.20 and CHF 36.70. The non-cash-relevant expenses for all unvested SAR and stock options in the reporting period per September 30, 2018, amounted to CHF 5.2 million (2017: CHF 5.5 million).

### 13.1 Share Appreciation Rights Plans

Santhera has established a Board Share Appreciation Plans (**BSARP**), the BSARP 2016, the BSARP 2017, for the members of its Board and Employee Share Appreciation Rights Plans (**ESARP**), the ESARP 2016 and the ESARP 2017, for the Executive Management, employees and consultants. SAR grants are made mainly periodically at the full discretion of the Board or as contractually agreed with employees. SARP introduced in 2017 foresee vesting of 1/3 of the SAR on the first anniversary; the remaining 2/3 vest each following quarter end through the second and third year after the grant date (8 times 1/12 of the SAR granted). In January 2018, Santhera has introduced ESARP 2018 in order to provide a special grant for the Executive Management and employees. Besides the usual terms, this grant contains an additional vesting condition, which is based on Santhera obtaining a positive opinion of the Committee for Medicinal Products for Human Use (**CHMP**) with respect to the marketing authorization of idebenone for the treatment of patients with DMD in the European Union (**EU**).

In the reporting period ended September 30, 2018, a total of 622,282 SARs with exercise prices between CHF 16.20 and CHF 36.70 were granted. In the same period ending September 30, 2017, a total of 316,986 SAR with exercise prices between CHF 54.85 and CHF 77.80 were granted.

#### Number of SAR outstanding

nine months ended September 30, number of SAR	<b>2018</b>	<b>2017</b>
<b>Outstanding at January 1</b>	<b>360,110</b>	<b>56,581</b>
Granted <sup>1</sup>	622,282	316,986
Exercised	0	0
Forfeited	-213,873	-1,231
Expired	0	0
<b>Outstanding September 30</b>	<b>768,519</b>	<b>372,336</b>

<sup>1</sup> The weighted average fair value of the SAR granted during the reporting period in 2018 was CHF 12.11 (in the comparative reporting period 2017 the weighted average fair value of SAR granted was CHF 27.18).

### 13.2 Stock Option Plans

Santhera has established Employee Stock Option Plans (**ESOP**), the ESOP 2010, the ESOP 2015, and Board Stock Option Plans (**BSOP**), the BSOP 2015, to align the long-term interests of the Board, the Executive Management and employees. Options granted under the stock option plans are equity-settled. No grants are made under ESOP and BSOP anymore.

In the reporting period ended September 30, 2018, no stock options were granted. In the same period ending September 30, 2017, no stock options were granted.

**Number of stock options outstanding**

nine months ended September 30, number of stock options	<b>2018</b>	<b>2017</b>
<b>Outstanding at January 1</b>	<b>288,442</b>	<b>313,365</b>
Granted	0	0
Forfeited	-19,643	-10,533
Expired	0	-753
Exercised	0	-5,300
<b>Outstanding at September 30</b>	<b>268,799</b>	<b>296,779</b>

**14 Related Party Transactions**

During the reporting period 2018, a total of 62,659 SAR were granted to members of the Board and 139,194 SAR were granted to members of the Executive Management. In the same period in 2017, a total of 15,120 SAR were granted to members of the Board and 104,033 SAR to members of the Executive Management.

**15 Subsequent Events**

On November 20, 2018, Santhera announced the signing of an agreement with Idorsia Pharmaceuticals Ltd, Allschwil, Switzerland (Idorsia). Under the terms of the agreement, Idorsia will grant Santhera the option to an exclusive sub-license for vamorolone in all indications and all territories except Japan and South Korea. Idorsia will receive as consideration for entering into the agreement 1,000,000 (one million) new registered shares from Santhera's existing authorized share capital and an upfront cash component of USD 20 million. Santhera may exercise the option upon receipt of data from the Phase IIb VISION-DMD study (VBP15-004) and following a one-time consideration to Idorsia of USD 30 million.

Following the exercise of the worldwide vamorolone license option by Idorsia and exercise of the vamorolone sub-license option for all territories worldwide except Japan and South Korea by Santhera, Santhera will pay to Idorsia regulatory and commercial milestone payments of up to USD 80 million in the DMD indication and four one-time sales milestone payments of up to USD 130 million in aggregate. Regulatory milestone payments by Santhera to Idorsia for three additional indications amount to up to USD 205 million in aggregate. Upon commercialization of vamorolone, Santhera has committed to pay tiered royalties ranging from a single-digit percentage to low double-digit percentage on the annual net sales of vamorolone to Idorsia.

In connection with the above announcement, Santhera informed that it intends to finance the cash payment through an ordinary capital increase.

In order to effect the upfront cash payment to Idorsia for the rights to vamorolone and to fund its ongoing activities, Santhera's Board of Directors has called an Extraordinary General Meeting (EGM) to be held on December 11, 2018, and is proposing to the EGM an ordinary capital increase of up to 3,500,000 registered Shares of the Company with a nominal value of CHF 1 each.

Santhera plans to raise approximately CHF 50 million of gross proceeds. The Company intends to use the net proceeds of the capital increase, together with available liquid funds, to finance the upfront cash payment to Idorsia, to further invest in the development of vamorolone and to fund ongoing business activities including the commercialization of Raxone® in Leber's hereditary optic neuropathy (LHON), market entry preparations for idebenone in DMD, and the development of POL6014 for the treatment of cystic fibrosis.

# Statutory Financial Statements of Santhera Pharmaceuticals Holding AG

## Contents

Balance Sheet .....	3
Income Statement .....	4
Notes to the Statutory Financial Statements .....	5
1 Introduction .....	5
2 Principles .....	5
3 Information on Balance Sheet and Income Statement Items .....	7
4 Other Information .....	9

## Balance Sheet

	in CHF thousands	Notes	Sep. 30, 2018
<b>Assets</b>			
Cash and cash equivalents			6,472
Financial assets short-term			5,735
Other receivables from third parties			94
Prepaid expenses and accrued income			202
Restricted cash short-term			3,000
<b>Current assets</b>			<b>15,503</b>
Loans to shareholdings		3.1	91,068
Investments in shareholdings		3.2	216
Restricted cash long-term			1,500
<b>Noncurrent assets</b>			<b>92,784</b>
<b>Total assets</b>			<b>108,287</b>
<b>Liabilities and equity</b>			
Trade accounts payable to third parties			37
Other accounts payable to third parties			43
Accrued expenses			730
<b>Current liabilities</b>			<b>810</b>
Senior unsecured convertible bonds <sup>1</sup>		2	60,000
<b>Noncurrent liabilities</b>			<b>60,000</b>
<b>Total liabilities</b>			<b>60,810</b>
Share capital		3.3	6,528
<i>Reserves from capital contributions</i>			450
<i>Other capital reserves</i>			2,916
Statutory capital reserves			3,366
<i>Accumulated result</i>			-18,767
<i>Results carried forward</i>			-13,752
<i>Net result for the period</i>			-5,015
<i>Other voluntary reserves (free reserves)</i>			57,495
Voluntary accumulated result and other reserves			38,728
Treasury shares		3.4	-1,145
<b>Total equity</b>			<b>47,477</b>
<b>Total liabilities and equity</b>			<b>108,287</b>

<sup>1</sup> interest bearing

## Income Statement

for the nine month period ended September 30, in CHF thousands	Notes	2018
Income from shareholdings	3.5	890
Other operating income		0
<b>Total operating income</b>		<b>890</b>
General and administrative expenses	3.6	-2,179
Employee costs		-952
Other operating expenses		-18
<b>Total operating expenses</b>		<b>-3,149</b>
<b>Operating result</b>		<b>-2,259</b>
Financial income		272
Financial expenses		-3,046
<b>Financial result</b>		<b>-2,774</b>
Reversal on allowance of investment		18
<b>Result before taxes</b>		<b>-5,015</b>
Direct taxes		0
<b>Net result</b>		<b>-5,015</b>

## Notes to the Statutory Financial Statements

### 1 Introduction

Santhera Pharmaceuticals Holding AG (the Company or Santhera) is the parent company of Santhera Group. The Company has its business offices at Hohenrainstrasse 24 in 4133 Pratteln, Switzerland.

### 2 Principles

#### General

The statutory financial statements of the Company are prepared in accordance with the general accepted accounting principles as set out in Art. 957 to Art. 963b, of the Swiss Code of Obligations (**CO**). Since Santhera prepares consolidated financial statements in accordance with International Financial Reporting Standards (**IFRS**) of the International Accounting Standards Board (**IASB**), a recognized accounting standard, the Company has, in accordance with the CO, elected to forego presenting the statement of cash flows, the additional disclosures and the management report otherwise required by the CO.

#### Material uncertainties and going concern

Santhera Group is subject to different risks and uncertainties, including but not limited to the uncertainty of the development of its clinical studies, regulatory approval and marketing activities in order to achieve profitability. The Group's ability to continue operations as planned for the next 12 months depends on cash flows from ongoing product sales, the results of its development activities and the capability to raise additional funds through an ordinary capital increase.

Santhera Group continues to generate increasing income from product sales for the indication Leber's hereditary optic neuropathy (**LHON**). Moreover, the Company has collected additional data in patients with Duchenne muscular dystrophy (**DMD**) needed for submission of a Marketing Authorization Application for this indication in the European Union (**EU**) in early 2019. Santhera also prepares for submission of a New Drug Application for patients with DMD in the United States. Lastly, Santhera Pharmaceuticals (Switzerland) AG entered into an option for a sub-licensing agreement with Idorsia for the steroid vamorolone (see note 4.6 "*Events after the reporting date*" for further discussion), which will require an upfront cash payment of USD 20 million within forty days after November 20, 2018.

Santhera Group's cash, cash equivalents and short-term financial assets amounted to CHF 25.4 million as of September 30, 2018. A material uncertainty remains as to whether Santhera Group's funding is sufficient to support its going concern for another twelve months.

In order to effect the upfront cash payment to Idorsia for the rights to vamorolone and to fund its ongoing activities, Santhera's Board of Directors has called an Extraordinary General Meeting (**EGM**) to be held on December 11, 2018, and is proposing to the EGM an ordinary capital increase of up to 3,500,000 registered Shares of the Company with a nominal value of CHF 1 each. Santhera plans to raise approximately CHF 50 million of gross proceeds (see note 4.6 "Events after the reporting date" for further discussion). Shareholders should note that whilst the Board and Executive Management continue to apply best efforts to raise additional funds, there is no guarantee that such funds can be raised. The availability of sufficient funds is crucial for Santhera and its ability to continue and grow its operations, including the financing of the upfront cash payment to Idorsia. Based on the Board's and the Executive Management's plan as discussed above, the Board of Directors is confident to ensure business continuation and meet its obligations for a further twelve months. Hence, the financial statements have been prepared on a going concern basis.

### **Cash**

Santhera holds cash balances, denominated mainly in Swiss francs (**CHF**) which include cash deposited in demand bank accounts, money market investment accounts and other liquid investments and interest earned on such cash balances.

### **Financial assets short-term**

Financial assets (units in a fund) are held for trading and measured at fair value. In case of gains and losses from such assets are recognized through the income statement as financial income or financial expense.

### **Current assets and liabilities**

Current assets are recorded at historical cost less adjustments for impairment of value and current liabilities at historical cost.

### **Loans to shareholdings**

These are valued at their acquisition cost adjusted for impairment losses.

### **Investments in shareholdings**

Investments in shareholdings are recorded at acquisition cost less adjustments for impairment of value. Investments in subsidiaries are evaluated for impairment annually and an impairment loss is recorded when the carrying amount of such assets exceeds the fair value. Fair value estimates of investments are predominantly based on the income approach.

### **Convertible bonds**

On February 17, 2017, Santhera issued senior unsecured convertible bonds in the nominal amount of CHF 60 million. The bonds, listed on the SIX, are interest bearing (5%) with a maximum term of 5 years and are convertible into registered Shares of Santhera with a nominal value of CHF 1 each. The initial conversion price was fixed at CHF 86.4006 and has been reset in accordance with the terms of the bond in February 2018 to CHF 64.80. In addition, Santhera may call the convertible bonds at any time on or after the second anniversary of the issue date at par, plus accrued interest, if any, if the VWAP of the Shares is at least 160% of the conversion price.

### Treasury shares

Treasury shares are recognized at acquisition cost and deducted from shareholders' equity at the time of acquisition. Santhera holds treasury shares for market making which is maintained by an external bank. In case of a resale, the gain or loss is recognized through the income statement as financial income or financial expenses.

### Related parties

In the meaning of the Swiss Accounting Law, related parties are only considered to be shareholders, direct and indirect subsidiaries (shareholdings) and the Board of Directors.

## 3 Information on Balance Sheet and Income Statement Items

### 3.1 Loans to shareholdings

Loans are granted to shareholdings primarily to fund the development and marketing activities of the Santhera Group (September 30, 2018: CHF 263.4 million). Until the end of 2015 the balance consisted of fully impaired and subordinated loans to Santhera Pharmaceuticals (Schweiz) AG. To finance the activities in development and the commercialization of LHON, starting 2016 the loan granted to Santhera Pharmaceuticals (Schweiz) AG was increased (with the additional loans also being subordinated). As part of the reassessment as of September 30, 2018, Executive Management concluded that approximately 35% of the total loan balance is recoverable considering a more positive outlook, both in terms of market success of the developed and launched product (Raxone in LHON) and the development progress in other indications (e.g. idebenone in DMD).

### 3.2 Investments in shareholdings

In 2018, the following companies are direct subsidiaries of Santhera Pharmaceuticals Holding AG (100% ownership and 100% voting rights):

	Share capital	Sep. 30, 2018
Santhera Pharmaceuticals (Schweiz) AG Pratteln, Switzerland	CHF	125,000
Santhera Pharmaceuticals (Deutschland) GmbH Lörrach, Germany	EUR	25,000
Santhera Pharmaceuticals (USA), Inc. Burlington, US	USD	1,000
Santhera Pharmaceuticals (Canada), Inc. Montréal, Canada	CAD	1,000
Oy Santhera Pharmaceuticals (Finland) Ltd Helsinki, Finland	EUR	2,500

Santhera Pharmaceuticals (Schweiz) AG is the primary operational entity while Santhera Pharmaceuticals (Deutschland) GmbH holds the market authorization for the EU. Oy Santhera Pharmaceuticals (Finland) Ltd is not employing any personnel.

The following companies are 100% direct subsidiaries (100% voting rights) of Santhera Pharmaceuticals (Schweiz) AG:

	Share capital	Sep. 30, 2018
Santhera Pharmaceuticals (Liechtenstein) AG Ruggell, Fürstentum Liechtenstein	CHF	50,000
Santhera (Italy) S.r.l. Milano, Italy	EUR	50,000
Santhera (Germany) GmbH München, Germany	EUR	50,000
Santhera (Netherlands) B.V. Nieuwegein, The Netherlands	EUR	50,000
Santhera (UK) Limited London, United Kingdom	GBP	50,000
Santhera Pharmaceuticals (Spain), S.L.U. Bilbao, Spain	EUR	50,000

### 3.3 Share capital

During 2018, the share capital was increased by a total amount of CHF 238,924 to CHF 6,527,479 as of September 30, 2018 (December 31, 2017: CHF 6,288,555) through the issuing of shares from the authorized share capital.

### 3.4 Treasury shares

The movement of treasury shares held by Santhera was as follows:

	No of Shares	TCHF
December 31, 2017	9,921	335
Purchase	128,080	3,049
Sale	-81,897	-2,239
September 30, 2018	56,104	1'145

### 3.5 Income from shareholdings

Income from shareholdings represents reimbursement for management services provided by the Company to its major shareholding Santhera Pharmaceuticals (Schweiz) AG.

### 3.6 General and administrative expenses

For the nine months, ended September 30, 2018, In CHF thousands	<b>1.-9.2018</b>
Administrative expenses	997
Consulting expenses	1,182
<b>Total</b>	<b>2,179</b>

---

## 4 Other Information

### 4.1 Full-time equivalents

The number of full-time equivalents at period end was not above 10 in 2018.

### 4.2 Significant shareholders (>2%)

Pursuant to information from the Company's share register and the disclosure of participations made to the Company in accordance with applicable stock exchange regulation, the following shareholders owned 2% or more of the Company's share capital as registered in the commercial register at September 30, 2018: 6,527,479 shares:

	<b>2018</b> <b>Shares<sup>1</sup></b>	<b>2018</b> <b>%</b>
Bertarelli Ernesto, Guichard-Bertarelli Donata and Bertarelli Maria-Iris, Switzerland <sup>3</sup>	536,278	8.2
Iglu Group, Switzerland <sup>3</sup>	533,350	8.2
Roderick Wong (RTW Master Fund, LTD, US)	325,638	5.0
JPMorgan Chase & Co., US	197,761	3.0
Polyphor AG, Switzerland	149,590	2.3

<sup>1</sup> Including disclosures until September 30, 2018

#### 4.3 Disclosure of shares and equity rights (share appreciation rights and stock options) held by members of the Board and Executive Management (and their respective related party)

As of September 30, 2018:

	Number of Shares	Number of vested equity rights	Number of un-vested equity rights	Total number of equity rights
<i>Board of Directors</i>				
Elmar Schnee, Chairman	2,000	21,207	1,870	23,077
Martin Gertsch, Vice-Chairman	38,109	22,026	5,623	27,649
Philipp Gutzwiller, Director	500	14,923	1,317	16,240
Thomas Meier, Director		--- See below ---		
Patrick Vink, Director	1,000	18,502	1,385	19,887
<i>Executive Management</i>				
Thomas Meier, CEO	75,562	39,953	22,833	62,786
Günther Metz, Head Business Development	0	24,905	22,252	47,157
Christoph Rentsch, Chief Financial Officer	0	35,096	21,803	56,899
Kristina Sjöblom Nygren, Chief Medical Officer & Head Development	0	36,937	0	36,937
Giovanni Stropoli, Chief Commercial Officer Europe & Rest of World until September 30, 2018 <sup>1</sup>	250	0	20,751	20,751
Oliver Strub, General Counsel and Secretary to the Board	0	25,472	14,498	39,970

<sup>1</sup> Number of Shares as of September 30, 2018

#### 4.4. Disclosure of the allocation of equity rights for Board of Directors, Executive Management and employees of Santhera Group

	1.-9.2018	2018
	Quantity	Value (in TCHF) <sup>1</sup>
Board of Directors	62,659	463
Executive Management	139,194	1,266
Employees of Santhera Group	420,429	5,809
<b>Total</b>	<b>622,282</b>	<b>7,538</b>

<sup>1</sup> Value of the equity rights calculated in accordance with the Hull-White model at the date of allocation in accordance with the terms of the award. The tax value of equity rights is 0 until they would be exercised. Such equity rights values are theoretical values and do not reflect income tax values and do also take into consideration certain vesting provisions.

# Consolidated Financial Statements

## Contents

Consolidated Balance Sheet .....	16
Consolidated Income Statement .....	17
Consolidated Statement of Comprehensive Income .....	18
Consolidated Cash Flow Statement .....	19
Consolidated Statement of Changes in Equity .....	20
Notes to the Consolidated Financial Statements .....	21
1 General Information .....	21
2 Summary of Significant Accounting Policies .....	21
3 Critical Accounting Estimates, Assumptions and Judgments .....	29
4 Exchange Rates of Principal Currencies .....	29
5 Tangible Assets .....	30
6 Intangible Assets .....	31
7 Prepaid Expenses and Other Assets .....	32
8 Inventories .....	32
9 Trade and Other Receivables .....	32
10 Cash and Cash Equivalents and Restricted Cash.....	33
11 Share Capital .....	33
12 Financial Assets and Liabilities .....	35
13 Deferred Taxes .....	37
14 Trade and Other Payables .....	38
15 Accrued Expenses .....	38
16 Commitments and Contingent Liabilities .....	38
17 Equity Rights Plans .....	40
18 Segment and Geographic Information .....	46
19 Other Operating Income .....	46

20	Operating Expenses by Nature .....	47
21	Employee Expenses and Benefits .....	47
22	Financial Income/Expenses .....	50
23	Income Taxes .....	51
24	Earnings/Loss per Share .....	52
25	Related Party Transactions .....	52
26	Risk Management Objectives and Policies .....	52
27	Events after the Reporting Date .....	56
	Statutory Auditor's Report on the Audit of the Consolidated Financial Statements .....	58

## Consolidated Balance Sheet

	As of December 31, in CHF thousands	Notes	2017	2016
<b>Assets</b>				
Tangible assets		5	2,157	517
Intangible assets		6	23,560	26,549
Financial assets long-term			713	270
Restricted cash long-term		10	4,500	0
Deferred tax assets		13	1,242	1,106
<b>Noncurrent assets</b>			<b>32,172</b>	<b>28,442</b>
Prepaid expenses and other assets		7	853	583
Inventories		8	10,147	7,676
Trade and other receivables		9	5,402	4,276
Financial assets short-term		12	13,011	0
Restricted cash short-term		10	3,000	0
Cash and cash equivalents		10	45,195	49,815
<b>Current assets</b>			<b>77,608</b>	<b>62,350</b>
<b>Total assets</b>			<b>109,780</b>	<b>90,792</b>
<b>Equity and liabilities</b>				
Share capital		11	6,289	6,280
Capital reserves and share premium			392,002	382,322
Retained earnings			-360,081	-308,549
Employee benefit reserve			-4,905	-4,734
Treasury shares		11	-335	-172
Translation differences			-714	-796
<b>Total equity</b>			<b>32,256</b>	<b>74,351</b>
Convertible bonds		12	53,111	0
Derivative financial instruments		12	2,792	0
Pension liabilities		21	8,375	6,183
<b>Total noncurrent liabilities</b>			<b>64,278</b>	<b>6,183</b>
Trade and other payables		14	4,734	4,458
Accrued expenses		15	8,512	5,800
<b>Total current liabilities</b>			<b>13,246</b>	<b>10,258</b>
<b>Total liabilities</b>			<b>77,524</b>	<b>16,441</b>
<b>Total equity and liabilities</b>			<b>109,780</b>	<b>90,792</b>

## Consolidated Income Statement

For the year ended December 31, in CHF thousands	Notes	2017	2016
Net sales	18	22,943	19,033
Cost of goods sold		-4,104	-3,883
<i>Of which amortization intangible assets</i>		-3,039	-3,039
Other operating income	19	270	361
Development	20	-26,561	-17,675
Marketing and sales	20	-28,522	-21,051
General and administrative	20	-14,416	-9,805
Other operating expenses	20	-64	-107
<b>Operating expenses</b>	<b>20</b>	<b>-69,563</b>	<b>-48,638</b>
<b>Operating result</b>		<b>-50,454</b>	<b>-33,127</b>
Financial income	22	4,134	928
Financial expenses	22	-4,955	-995
<b>Result before taxes</b>		<b>-51,275</b>	<b>-33,194</b>
Income taxes	23	-257	-2,221
<b>Net result</b>		<b>-51,532</b>	<b>-35,415</b>
Basic earnings/loss per share (in CHF)	24	-8.22	-5.65
Diluted earnings/loss per share (in CHF)	24	-8.22	-5.65

## Consolidated Statement of Comprehensive Income

For the year ended December 31, in CHF thousands	Notes	2017	2016
<b>Net result</b>		<b>-51,532</b>	<b>-35,415</b>
<i>Items never to be reclassified to net income in subsequent periods:</i>			
Actuarial gains/losses on defined benefit plans	21	-171	-1,776
<i>Items to be reclassified to net income in subsequent periods:</i>			
Currency translation differences		82	-18
<b>Other comprehensive result</b>		<b>-89</b>	<b>-1,794</b>
<b>Total comprehensive result</b>		<b>-51,621</b>	<b>-37,209</b>

## Consolidated Cash Flow Statement

For the year ended December 31, in CHF thousands	Notes	2017	2016
Result before taxes		-51,275	-33,194
Depreciation of tangible assets	5	257	168
Amortization of intangible assets	6	3,125	3,096
Expenses for equity rights plans	17, 20	9,687	4,683
Change in fair value of derivatives		-2,540	0
Change in fair value of financial assets short-term		-96	0
Change in pension liabilities	21	2,021	450
Taxes paid		-392	-266
Change in net working capital		315	-2,131
Total financial result	22	821	67
Interest received	22	5	5
Interest paid	22	-1,561	-15
<b>Cash flow from operating activities</b>		<b>-39,633</b>	<b>-27,137</b>
Investments in tangible assets	5	-1,261	-289
Investments in intangible assets	6	-136	-86
Investments in other short-term financial assets	12	-12,915	0
Investments in other long-term financial assets		-427	-84
Change in restricted cash	10	-7,500	0
<b>Cash flow from investing activities</b>		<b>-22,239</b>	<b>-459</b>
Capital increases from options exercised	11	34	385
Proceeds from sale of treasury shares	11	9,372	418
Purchase of treasury shares	11	-9,567	-172
Proceeds from convertible bonds	12	57,269	0
<b>Cash flow from financing activities</b>		<b>57,108</b>	<b>631</b>
Effects of exchange rate changes on cash and cash equivalents		144	-79
<b>Net increase/decrease in cash and cash equivalents</b>		<b>-4,620</b>	<b>-27,044</b>
Cash and cash equivalents at January 1		49,815	76,859
<b>Cash and cash equivalents at December 31</b>		<b>45,195</b>	<b>49,815</b>

## Consolidated Statement of Changes in Equity

In CHF thousands	Notes	Share capital	Capital reserves and share premium	Retained earnings	Employee benefit reserve	Treasury shares	Translation differences	Total
<b>Balance at January 1, 2016</b>		<b>6,263</b>	<b>377,031</b>	<b>-273,134</b>	<b>-2,958</b>	<b>-177</b>	<b>-778</b>	<b>106,247</b>
Net result		0	0	-35,415	0	0	0	-35,415
Other comprehensive result	21	0	0	0	-1,776	0	-18	-1,794
<b>Total comprehensive result for the period</b>		<b>0</b>	<b>0</b>	<b>-35,415</b>	<b>-1,776</b>	<b>0</b>	<b>-18</b>	<b>-37,209</b>
Transactions for equity rights plans	17, 20	0	4,682	0	0	0	0	4,682
Capital increase from options exercise	11	17	368	0	0	0	0	385
Change in treasury shares		0	241	0	0	5	0	246
<b>Balance at December 31, 2016</b>		<b>6,280</b>	<b>382,322</b>	<b>-308,549</b>	<b>-4,734</b>	<b>-172</b>	<b>-796</b>	<b>74,351</b>
<b>Balance at January 1, 2017</b>		<b>6,280</b>	<b>382,322</b>	<b>-308,549</b>	<b>-4,734</b>	<b>-172</b>	<b>-796</b>	<b>74,351</b>
Net result		0	0	-51,532	0	0	0	-51,532
Other comprehensive result	21	0	0	0	-171	0	82	-89
<b>Total comprehensive result for the period</b>		<b>0</b>	<b>0</b>	<b>-51,532</b>	<b>-171</b>	<b>0</b>	<b>82</b>	<b>-51,621</b>
Transactions for equity rights plans	17, 20	0	9,687	0	0	0	0	9,687
Capital increase from options exercise	11	9	25	0	0	0	0	34
Change in treasury shares	11	0	-32	0	0	-163	0	-195
<b>Balance at December 31, 2017</b>		<b>6,289</b>	<b>392,002</b>	<b>-360,081</b>	<b>-4,905</b>	<b>-335</b>	<b>-714</b>	<b>32,256</b>

# Notes to the Consolidated Financial Statements

## 1 General Information

Santhera Pharmaceuticals Holding AG (the **Company**, together with its subsidiaries **Santhera** or **Group**) is a Swiss specialty pharmaceutical company focused on the development and commercialization of products for the treatment of neuro-ophthalmological, neuromuscular and pulmonary diseases, areas which include many orphan and niche indications with high unmet medical need.

The Company, having its primary listing of its registered shares (**Shares**) on the SIX Swiss Exchange (**SIX**), is a Swiss stock corporation and the parent company of the Group. Its purpose is to acquire, dispose and manage investments. The Company has its business offices at Hohenrainstrasse 24 in 4133 Pratteln, Switzerland. The legal domicile will remain at Hammerstrasse 49 in 4410 Liestal, Switzerland, until the forthcoming Annual General Meeting when the shareholders will vote on its relocation to Pratteln.

The consolidated financial statements were approved for publication by the Board of Directors (**Board**) on March 19, 2018. They are subject to approval by the Annual General Meeting of Shareholders (**AGM**) on April 12, 2018.

## 2 Summary of Significant Accounting Policies

The principal accounting policies applied in the preparation of these financial statements are set out below. These policies have been consistently applied to all the years presented, unless otherwise stated.

### Basis of preparation

The consolidated financial statements of Santhera have been prepared in accordance with International Financial Reporting Standards (**IFRS**).

The consolidated financial statements are based on the financial statements of the individual Santhera companies prepared for the same reporting period using consistent accounting policies. The consolidated financial statements are prepared using the historical cost convention except for the re-valuation to fair value of certain financial assets and financial liabilities.

The presentation currency is Swiss francs (**CHF**). All figures included in these financial statements and notes to the financial statements are rounded to the nearest CHF 1,000 except where otherwise indicated.

### Consolidation

Subsidiaries in which the Company has a direct or indirect controlling interest are consolidated. Control exists when the investor is exposed, or has rights, to variable returns from its investment with the investee and has the ability to affect those returns through its power over the investee. Control is normally evidenced when the Company owns, either directly or indirectly, more than 50% of the voting rights or potential voting rights of a company's share capital that are currently exercisable.

The consolidated financial statements of Santhera include the accounts of Santhera Pharmaceuticals Holding AG, Liestal, Switzerland, and its wholly owned subsidiaries Santhera Pharmaceuticals (Schweiz) AG, Pratteln, Switzerland; Santhera Pharmaceuticals (USA), Inc., Burlington, US; Santhera Pharmaceuticals (Canada), Inc., Montréal, Canada; Santhera Pharmaceuticals (Deutschland) GmbH, Lörrach, Germany; and Oy Santhera Pharmaceuticals (Finland) Ltd, Helsinki, Finland. The accounts further include the wholly owned subsidiaries of Santhera Pharmaceuticals (Schweiz) AG: Santhera Pharmaceuticals (Liechtenstein) AG, Ruggell, Fürstentum Liechtenstein; Santhera (Italy) S.r.l., Milano, Italy; Santhera (Germany) GmbH, München, Germany; Santhera (Netherlands) B.V., Nieuwegein, The Netherlands; and Santhera (UK) Limited, London, United Kingdom.

Consolidation commences from the date on which control is transferred to the Company, and subsidiaries are no longer consolidated from the date that control ceases. Intercompany balances and transactions between Group companies are eliminated. Intercompany transactions solely result from providing services, financing and selling goods to other Group companies.

### **Changes in accounting policies**

#### *New, revised or amended IFRS standards and interpretations 2017*

The following new, revised or amended standards that became effective on January 1, 2017 did not have any significant impact on the consolidated financial statements.

- IAS7 Statement of Cash Flows: Disclosure Initiative  
The amendments require entities to provide disclosure of changes in their liabilities arising from financing activities, including both changes arising from cash flows and non-cash changes.
- IAS 12 Income Taxes (Amendments) Recognition of Deferred Tax Assets for Unrealized Losses
- Annual Improvements to IFRSs 2014–2016 Cycle

#### *New, revised or amended IFRS standards and interpretations issued but not yet effective*

The following new, revised or amended standards have been published but are not yet effective and have not been early adopted by the Group.

- IFRS 9, Financial Instruments (effective January 1, 2018)  
IFRS 9 introduces a single approach for the classification and measurement of financial assets according to their cash flow characteristics and the business model they are managed in, and provides a new impairment model based on expected credit losses. IFRS 9 also includes new regulations regarding the application of hedge accounting to better reflect an entity's risk management activities especially with regard to managing nonfinancial risks. The Group adopted the new standard on the required effective date as of January 1, 2018, and did not restate comparative information. During 2017, the Group has performed an impact assessment of all three aspects of IFRS 9. Upon implementation of IFRS 9, the assessment did not show a significant impact.

- IFRS 9 requires the Group to record expected credit losses on all of its trade receivables, either on a 12-month or lifetime basis. The Group applies the simplified approach and records lifetime expected losses.
- IFRS 15 Revenue from Contracts with Customers (effective January 1, 2018)  
According to the new standard, revenue is recognized to depict the transfer of promised goods or services to a customer in an amount that reflects the consideration to which the Company expects to be entitled to in exchange for those goods or services. Revenue is recognized when, or as the customer obtains control of the goods or services.  
The Group is focused on the development and commercialization of products for the treatment of mitochondrial and neuromuscular diseases. It has identified the sale of product as the only performance obligation and revenue stream from its contracts with customers. For these contracts the adoption of IFRS 15 did not have any impact on the Group's revenue and profit or loss and no transition adjustment was recorded upon adoption as of January 1, 2018.
- IFRS 16 Leases (effective January 1, 2019)  
The new standard eliminates the current classification model for lessee's lease contracts as either operating or finance leases and, instead, introduces a single lessee accounting model requiring lessees to recognize right-of-use assets and lease liabilities for leases with a term of more than twelve months. This brings the previous off-balance sheet leases on the balance sheet in a manner largely comparable to current finance lease accounting. A lessee can choose to apply the standard using either a full retrospective or a modified retrospective approach. Adoption of IFRS 16 will result in the Group recognizing right of use assets and lease liabilities for all contracts that are, or contain, a lease. For leases currently classified as operating leases, under current accounting requirements the Group does not recognize related assets or liabilities, and instead spreads the lease payments on a straight-line basis over the lease term, disclosing in the notes to its annual consolidated financial statements the total commitment. The Group is expecting that current leasing arrangements relating to real estate and cars will be capitalized under IFRS 16. In 2018, the Group will continue to assess the potential effect of IFRS 16 on its consolidated financial statements.  
The Group does not expect any other standards issued by the IASB, but not yet effective, to have a material impact on the Group's financial statements.

## Segment reporting

Santhera has one operating segment, namely the development and commercialization of products for the treatment of neuro-ophthalmological, neuromuscular and pulmonary diseases. The Board, the Executive Management and senior managers, being the Chief Operating Decision Makers (**CODM**), assess the reporting data and allocate resources as one segment on a consolidated level according to operating expenses by function. Santhera generates revenue from sales of Raxone for the treatment of LHON. Geographic revenue information is based on location of the customer or licensee.

## Foreign currency translations

The consolidated financial statements are presented in CHF. The functional currency of each of Santhera's companies is the currency of the primary economic environment in which the local entity

operates. Transactions in foreign currencies are accounted for at the rates prevailing at the dates of the transaction. Translation differences from financial transactions are included in the financial result.

Gains and losses resulting from the translation of foreign currency transactions and from the adjustment of foreign currency monetary assets and liabilities at the reporting date are recognized in the income statement.

Assets and liabilities of foreign entities are translated into CHF using the balance sheet exchange rates at year-end. Income and expenses are translated into CHF at average exchange rates. The exchange differences arising on the retranslation are accounted for in the statements of comprehensive income/equity.

### **Intangible assets**

Patents, licenses, trademarks and other intangible assets are capitalized as intangible assets when it is probable that future economic benefits will be generated. Such assets are in general amortized on a straight-line basis over their useful lives. Estimated useful life is the lower of legal duration or economic useful life. The estimated useful life of the intangible assets is regularly reviewed and if necessary, the future amortization charge is accelerated. For pharmaceutical products, the estimated useful life normally corresponds to the remaining lifetime of their patent or orphan drug protection (up to 20 years).

#### **IT software**

Acquired IT software licenses are capitalized on the basis of the costs incurred to acquire and implement the specific software. These costs are amortized on a straight-line basis over their estimated useful lives (2 to 5 years).

### **Tangible assets**

Tangible assets are stated at cost less accumulated depreciation and any impairment losses. Depreciation is calculated on a straight-line basis over the estimated useful life of the asset or the shorter lease term, as follows:

	Useful life
Equipment	4 to 10 years
IT hardware	2 to 5 years
Leasehold improvements	2 to 10 years

### **Impairment of assets**

Assets include intangible assets not yet available for use, intangible assets with finite useful lives and tangible assets. In general, and in accordance with the terms of IFRS, assets not in use are capitalized at cost in the balance sheet and reviewed for impairment at least annually. Impairment testing is performed at the same time every year or whenever there is an indication that the asset may be impaired. Once an intangible asset starts to be used, amortization starts. Testing for indicators of impairment is done at the end of each reporting period.

### **Trade and other receivables**

Receivables which generally have 30 to 60 days payment terms are stated at their nominal value less an allowance for any uncollectible amount if required. An allowance for doubtful debts is made when collection is deemed no longer probable.

### **Inventories**

Inventories are stated at the lower of cost or net realizable value using the weighted average cost formula.

### **Financial assets**

Generally, Santhera classifies its financial assets in the following categories:

#### *Financial assets at fair value through profit or loss*

This category has two subcategories: financial assets held for trading and those designated at fair value through profit or loss upon initial recognition. A financial asset is classified in this category if acquired principally for the purpose of selling in the short-term. Assets in this category are classified as current assets if they are either held for trading or are expected to be realized within 12 months of the reporting date. Valuation is at fair value through profit or loss. Financial assets at fair value through profit or loss are subsequently carried at fair value. Realized and unrealized gains and losses arising from changes in the fair value are included in the income statement in the period in which they arise.

#### *Loans and receivables*

Loans and receivables are nonderivative financial assets with fixed or determinable payments that are not quoted in an active market. They arise when Santhera provides money, goods or services directly to a debtor with no intention of trading the receivable. They are included in current assets, except for maturities longer than 12 months after the balance sheet date. These are classified as noncurrent assets. Loans and receivables are measured at amortized cost using the effective interest method.

### **Leases**

Leases of assets under which Santhera essentially assumes all the rewards and risks of ownership are classified as finance leases. Finance leases are capitalized as assets and liabilities at the commencement of the lease at the fair value of the leased item or, if lower, at the present value of the minimum lease payments. The assets acquired under these contracts are depreciated over the shorter of the estimated useful life of the asset or the lease term.

Leases of assets under which the risks and rewards of ownership are effectively retained by the lessor are classified as operating leases, and payments made are charged to the income statement on a straight-line basis.

### **Cash and cash equivalents**

This item includes cash on hand and at banks, deposits held at call with banks and other short-term highly liquid investments with original maturities of three months or less.

### **Restricted cash**

Cash set aside in escrow and not available to finance Santhera's day-to-day operations is shown under this category. Maturities of less than 12 months are considered short-term; those of more than 12 months are long-term.

### **Share capital**

Common shares are classified as equity. Incremental costs directly attributable to the issue of new common shares or options are shown in equity in the capital reserves and share premium as a deduction, net of tax, from the proceeds.

### **Treasury shares**

Treasury shares are purchased at cost and recognized as deduction from equity. Income or loss from subsequent sale is presented in equity.

### **Financial liabilities**

Santhera classifies its financial liabilities into two categories:

#### *Financial liabilities at fair value through profit or loss*

This category includes derivatives with negative replacement values. They are initially recognized at their fair value. Any subsequent change in fair value is recognized in the income statement in the period the changes occur.

#### *Other liabilities measured at amortized costs*

This category principally covers debt instruments and trade and other payables. They are initially recognized at fair value and subsequently measured at amortized costs using the effective interest method. Any difference between the net proceeds received and the principal value due on redemption is amortized over the duration of the debt instrument and is recognized as part of interest expense in the income statement.

### **Income taxes**

The income tax charge is based on profit for the year and includes deferred taxes. Deferred taxes are calculated using the liability method. Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Deferred tax assets and liabilities are measured using the tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled based on enacted or substantially enacted tax rates as of the balance sheet date.

The amount of deferred tax liabilities and deferred tax assets reflects the tax consequences on the balance sheet date of the Company's expectation of recovery or settlement of such carrying amount of its assets and liabilities.

Deferred tax assets and liabilities are not discounted and are classified as noncurrent assets (liabilities) in the balance sheet. They are offset against each other if they relate to the same taxable entity and tax authority.

Deferred tax assets are recognized when it is probable that sufficient taxable profits will be available against which the deferred tax assets can be utilized. At each balance sheet date, the Company reassesses unrecognized deferred tax assets and the carrying amount of deferred tax assets. The Company recognizes a previously unrecognized deferred tax asset to the extent that it has become probable that future taxable profit will allow the deferred tax asset to be recovered. The Company conversely reduces the carrying amount of a deferred tax asset to the extent that it is no longer probable that sufficient taxable profit will be available to allow the benefit of part or the entire deferred tax asset to be utilized. Deferred tax is provided on temporary differences arising on investments in subsidiaries, associates and joint ventures, except where the timing of the reversal of the temporary difference can be controlled and it is probable that the difference will not reverse in the foreseeable future.

### **Earnings/loss per share**

Basic earnings/loss per share are calculated by dividing the net profit/loss attributable to owners of ordinary Shares of the Company by the weighted average number of Shares outstanding during the reporting period. Diluted earnings per share are calculated by dividing the net profit attributable to owners of ordinary Shares of the Company by the weighted average number of shares issued and outstanding during the reporting period adjusted for Shares held as treasury shares (purchased at market), the number of potential shares from stock option plans and the convertible bonds.

### **Employee benefits**

#### *Post-retirement benefits*

Santhera operates both defined benefit and defined contribution pension schemes.

- **Defined benefit scheme:**

Santhera's pension plan in Switzerland is classified as a defined benefit plan. Payments under this scheme are made directly to the pension fund for the account of each insured person. Typically, on retirement, an employee will receive an amount of the accumulated defined benefit obligation depending on several factors such as the total individual amount paid in, age and implied life expectancy. The compensation will be in the form of a lifelong pension or a lump sum payment. The scheme also covers disability as a consequence of illness and death-in-service.

The liability recognized in the balance sheet in respect of defined benefit pension plans is the present value of the defined benefit obligation at the balance sheet date less the fair value of plan assets, adjusted for the effects of the asset ceiling, when relevant.

The defined benefit obligation is calculated annually by independent actuaries using the projected unit credit method. The present value of the defined benefit obligation is determined by discounting the estimated future cash outflows using interest rates of high-quality corporate bonds that are denominated in the currency in which the benefits will be paid and that have terms to maturity approximating the terms of the related pension liability.

- **Defined contribution schemes:**

Defined contribution schemes are also funded through direct payments for the account of each insured person. Upon retirement, an employee will receive an amount of the accumulated contributions in the form of a lifelong pension or a lump sum payment. No further obligations arise from these schemes other than the fixed periodic contributions to the plan.

### *Share-based compensation*

Santhera has established stock option and share appreciation rights (**SAR**) plans to align the long-term interests of the members of the Board, the Executive Management, employees and selected consultants who are eligible to participate. Under all plans, options and share appreciation rights are equity-settled. The fair value of options and SAR is determined at the grant date and recognized as personnel expense over the period Santhera receives services for each award. Where stock option awards are modified as a minimum, the expenses are recognized as if no terms had been modified; modifications which increase the fair value of options are expensed additionally. Unless determined otherwise by the Board, terminations of employment by the employer are treated as forfeiture and any previously accumulated share-based payment expenses for unvested awards are reversed.

### **Provisions**

Provisions are recognized when Santhera has a present obligation (legal or constructive) as a result of a past event, where it is more probable than not that a cash outflow will be required to fulfill the obligation and where a reliable estimate can be made of the amount of the obligation.

If the effect of the time value of money is material, provisions are determined by discounting the expected future outflows.

### **Revenue recognition**

Revenue comprises the fair value of the sale of goods and services, net of value-added tax, rebates, discounts, returns and after eliminating intercompany sales. Revenue is recognized when title, risks and rewards of the products are transferred to customers.

#### *Revenue from outlicensing*

Outlicensing agreements are concluded with third parties, where the counterparty has to pay license fees. In situations where no further performance commitment exists, revenue is recognized on the earlier of when payments are received or collection is assured. Where continuous involvement for a certain period is required in the form of technology transfer or technical support, revenues are recognized over the period in question.

#### *Revenue associated with up-front payments or performance milestones*

Such revenue is recognized in accordance with respective agreements.

#### *Revenue from royalties*

Royalty payments are recognized on an accrual basis in accordance with the respective agreements.

#### *Interest income*

Interest income is recognized on a pro rata temporis basis using the effective interest method.

### **Development / intangible assets**

Development expenses are charged to the income statement as incurred. They are capitalized as intangible assets when it is probable that future economic benefits will flow to Santhera. Such intangible assets are amortized on a straight-line basis over the period of the expected benefit when the asset becomes available for use, and are reviewed for impairment indicators at each balance sheet date. Assets not available for use are tested annually for impairment.

### 3 Critical Accounting Estimates, Assumptions and Judgments

The preparation of financial statements in conformity with IFRS requires the use of certain critical accounting estimates. It also requires management to exercise its judgment in the process of applying Santhera's accounting policies. Santhera makes estimates and assumptions concerning the future. The resulting accounting will not necessarily equal the related actual outcome. The following areas involve assumptions and estimates that can have a significant impact on the consolidated financial statements:

- Assessment of the Group's ability to continue as a going concern.
- Measurement and impairment testing of intangible assets.
- Measurement and testing for net realizable value of inventory, see note 8 "*Inventories*".
- Valuation of derivative financial instruments in connection with the convertible bonds, see note 12 "*Financial Assets and Liabilities*".
- Personnel expenses from share-based payments in accordance with IFRS 2, i.e. estimates regarding the valuation of equity rights plans when granted, see note 17 "*Equity Rights Plans*".
- Actuarial valuations in the context of defined benefit pension plans where various assumptions on e.g. discount rates, salary increase rates and mortality rates, etc. bear significant uncertainties due to the long-term nature of the plans, see note 21 "*Employee Expenses and Benefits*".

### 4 Exchange Rates of Principal Currencies

	Income statement in CHF average rates		Balance sheet in CHF year-end rates	
	2017	2016	2017	2016
1 Euro (EUR)	1.1114	1.0902	1.1691	1.0737
1 US dollar (USD)	0.9847	0.9851	0.9753	1.0160
1 British pound (GBP)	1.2683	1.3352	1.3173	1.2498
1 Canadian dollar (CAD)	0.7590	0.7435	0.7777	0.7532

## 5 Tangible Assets

	In CHF thousands	Equipment	IT hardware	Leasehold improvements	2017
<b>Cost</b>					
At January 1		237	756	91	1,084
Additions		114	396	1,376	1,886
Disposals		-2	-47	0	-49
Exchange differences		8	6	2	16
Reclassification		5	-5	0	0
<b>At December 31</b>		<b>362</b>	<b>1,106</b>	<b>1,469</b>	<b>2,937</b>
<b>Accumulated depreciation</b>					
At January 1		179	344	44	567
Additions		32	215	10	257
Disposals		-2	-47	0	-49
Exchange differences		2	3	0	5
Reclassification		1	-1	0	0
<b>At December 31</b>		<b>212</b>	<b>514</b>	<b>54</b>	<b>780</b>
<b>Net book value</b>		<b>150</b>	<b>592</b>	<b>1,415</b>	<b>2,157</b>

	In CHF thousands	Equipment	IT hardware	Leasehold improvements	2016
<b>Cost</b>					
At January 1		225	567	45	837
Additions		17	226	46	289
Disposals		-3	-36	0	-39
Exchange differences		-1	-2	0	-3
Reclassification		-1	1	0	0
<b>At December 31</b>		<b>237</b>	<b>756</b>	<b>91</b>	<b>1,084</b>
<b>Accumulated depreciation</b>					
At January 1		166	231	42	439
Additions		16	150	2	168
Disposals		-3	-36	0	-39
Exchange differences		0	-1	0	-1
<b>At December 31</b>		<b>179</b>	<b>344</b>	<b>44</b>	<b>567</b>
<b>Net book value</b>		<b>58</b>	<b>412</b>	<b>47</b>	<b>517</b>

## 6 Intangible Assets

	In CHF thousands	Idebenone	Fipamezole	IT software/ patents	2017
<b>Cost</b>					
At January 1		30,387	3,918	535	34,840
Additions		0	0	136	136
Disposals		0	0	-17	-17
<b>At December 31</b>		<b>30,387</b>	<b>3,918</b>	<b>654</b>	<b>34,959</b>
<b>Accumulated amortization</b>					
At January 1		4,052	3,918	321	8,291
Additions		3,039	0	86	3,125
Disposals		0	0	-17	-17
<b>At December 31</b>		<b>7,091</b>	<b>3,918</b>	<b>390</b>	<b>11,399</b>
<b>Net book value</b>		<b>23,296</b>	<b>0</b>	<b>264</b>	<b>23,560</b>
<b>2016</b>					
	In CHF thousands	Idebenone	Fipamezole	IT software/ patents	2016
<b>Cost</b>					
At January 1		30,387	3,918	477	34,782
Additions		0	0	86	86
Disposals		0	0	-28	-28
<b>At December 31</b>		<b>30,387</b>	<b>3,918</b>	<b>535</b>	<b>34,840</b>
<b>Accumulated amortization</b>					
At January 1		1,013	3,918	292	5,223
Additions		3,039	0	57	3,096
Disposals		0	0	-28	-28
<b>At December 31</b>		<b>4,052</b>	<b>3,918</b>	<b>321</b>	<b>8,291</b>
<b>Net book value</b>		<b>26,335</b>	<b>0</b>	<b>214</b>	<b>26,549</b>

During 2017 there was no trigger for impairment of intangible assets. "Idebenone" represents the main intangible asset of Santhera. It has become available for use in September 2015 and has an estimated useful life of 10 years. Prior to that it was not available for use and did not generate cash inflows.

## 7 Prepaid Expenses and Other Assets

	In CHF thousands	2017	2016
Prepayments		853	487
Other assets		0	96
<b>Total at December 31</b>		<b>853</b>	<b>583</b>

## 8 Inventories

	In CHF thousands	2017	2016
Raw material (active pharmaceutical ingredients)		7,488	5,052
Semi-finished goods		2,335	2,369
Finished goods		324	255
<b>Total at December 31</b>		<b>10,147</b>	<b>7,676</b>

## 9 Trade and Other Receivables

	In CHF thousands	2017	2016
Trade receivables		4,194	3,412
Other receivables		1,208	864
<b>Total at December 31</b>		<b>5,402</b>	<b>4,276</b>

Trade receivables in 2017 result from product sales, see note 18 *"Segment and Geographic Information"*. Other receivables consist mainly of amounts due from the government for tax reimbursements (e.g. VAT). They are due within 30 to 120 days and bear no interest. As of December 31, 2017, an allowance for doubtful debts of TCHF 55 was recognized on the trade receivables (no allowance was booked as of December 31, 2016).

## 10 Cash and Cash Equivalents and Restricted Cash

### 10.1 Cash and cash equivalents

	In CHF thousands	2017	2016
Cash at banks and on hand			
In CHF		34,730	44,358
In EUR		8,152	4,661
In GBP		697	546
In USD		1,496	149
In CAD		120	67
Other currencies		0	34
<b>Total at December 31</b>		<b>45,195</b>	<b>49,815</b>
<hr/>			
Of which: Short-term deposits			
In CHF		21,007	31,000

### 10.2 Restricted cash

	in CHF thousands	Dec. 31, 2017	Dec. 31, 2016
Long-term		4,500	0
Short-term		3,000	0
<b>Total at period end</b>		<b>7,500</b>	<b>0</b>

Restricted cash is designated for interest payments due related to the convertible bonds during the first 3 years (starting 2017). These funds are kept in an escrow account with the bond agent.

## 11 Share Capital

### Ordinary share capital

As of January 1, 2016, the share capital amounted to CHF 6,262,798, divided into 6,262,798 shares ("Shares") at a nominal value of CHF 1 each. During 2016, 17,059 Shares were issued from conditional capital upon the exercise of stock options. As a result, as of December 31, 2016, the share capital amounted to CHF 6,279,857, divided into 6,279,857 Shares at a nominal value of CHF 1 each.

During 2017, 8,698 Shares were issued from conditional capital upon the exercise of stock options. As a result, as of December 31, 2017, the share capital amounted to CHF 6,288,555, divided into 6,288,555 Shares at a nominal value of CHF 1 each.

## Treasury shares

In the second half of 2016, Santhera entered into an agreement for market making with a well-known bank. Independently, the bank buys and sells Shares on the market on behalf of the Company. On December 31, 2017, Santhera held 9,921 treasury Shares (2016: 3,616 treasury Shares).

## Authorized share capital

On the occasion of the AGM on May 11, 2016, the shareholders approved the increase of the authorized share capital as well as an extension. The Board is authorized to increase the share capital at any time until May 10, 2018, through the issuance of up to 1,500,000 Shares with a nominal value of CHF 1 each. An increase in instalments is permitted. For each such increase, the Board has to determine the issue price, the type of payment, the date of issuance of new Shares, the conditions for the exercise of pre-emptive rights and the beginning date for dividend entitlement.

## Conditional share capital

At the AGM on April 4, 2017, the shareholders approved a maximum increase of the share capital by an aggregate amount of CHF 700,000 (2016: CHF 550,000) through the issuance of a maximum of 700,000 (2016: 550,000) Shares with a nominal value of CHF 1 each. The Shares can be issued through the exercise of equity rights which are granted according to respective regulations of the Board.

In addition, the shareholders approved a maximum increase of the share capital by an aggregate amount of CHF 930,000 (2016: CHF 650,000) through the issuance of a maximum of 930,000 (2016: 650,000) Shares with a nominal value of CHF 1 per Share by the exercise of option and/or conversion rights which can be granted in connection with the issuance of bonds, similar obligations or other financial instruments by the Company or another Group company, and/or by the exercise of options which are granted by the Company or another Group company. In the case of the issue of bonds, similar obligations or other financial instruments linked with option and/or conversion rights, and in the case of the issue of option rights, the pre-emptive right of shareholders is excluded.

As of December 31, 2017, the Company had a conditional share capital, pursuant to the above provisions, whereby the share capital may be increased by

- a maximum amount of CHF 691,302 (2016: CHF 532,941) through the issuance of up to 691,302 (2016: 532,941) Shares, under the exclusion of shareholders' pre-emptive rights, for option rights being exercised under the Company's stock option plans, see note 17 "*Equity Rights Plans*", and
- a maximum amount of CHF 930,000 (2016: CHF 650,000) by issuing up to 930,000 (2016: 650,000) Shares, through the exercise of warrants/options and/or notes granted in connection with bonds or similar debt instruments linked with option and/or conversion rights granted by the Company.

## 12 Financial Assets and Liabilities

Santhera measures certain financial instruments at fair value. Fair values are categorized into the following hierarchy based on the inputs used to measure them:

- Level 1: Quoted prices (unadjusted) in active markets for identical assets or liabilities.
- Level 2: Inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly (as prices) or indirectly (derived from prices).
- Level 3: Unobservable inputs for the asset or liability. These inputs reflect the best estimates of Santhera based on criteria that market participants would use to determine prices for assets or liabilities at the reporting date.

### *12.1 Financial assets short-term*

Financial assets (units in a fund) are classified as held for trading. They are measured at fair value through profit or loss and based on quoted prices (Level 1). A net gain of TCHF 96 resulted during the reporting period (no such financial assets were held during the same period in 2016).

### *12.2 Financial liabilities*

On February 17, 2017, Santhera issued senior unsecured convertible bonds in the nominal amount of CHF 60 million. Transaction costs of CHF 2.7 million lead to a net amount of CHF 57.3 million (consisting of senior unsecured convertible bonds (CHF 52.0 million) and embedded derivative financial instruments (CHF 5.3 million)). The bonds, listed on the SIX, are interest bearing (5%) with a maximum term of 5 years and are convertible into registered Shares of Santhera with a nominal value of CHF 1 each. The initial conversion price is fixed at CHF 86.4006 and will be reset after the first year if the volume weighted average price (**VWAP**) of the Shares during a specified period of time will be below the reference share price (CHF 71.9969). In February 2018, the conversion price was reset to CHF 64.80. In addition, Santhera may call the convertible bonds at any time on or after the second anniversary of the issue date at par, plus accrued interest, if any, if the VWAP of the Shares is at least 160% of the conversion price. The convertible bonds are measured at amortized costs applying the effective interest method. The fair value of the bond at December 31, 2017, amounts to CHF 51.6 million (no convertible bonds in 2016).

The embedded financial derivatives (conversion right, reset mechanism and early redemption option) are valued by an independent consultant initially and at period end at fair value, applying a simulation-based valuation approach. The valuation of the embedded derivatives is based on input parameters, classified as Level 3. The simulation is mainly based on the historical volatility of Santhera shares. The period of volatility data used is measured according to the remaining life of the convertible bonds. The volatility used as per December 31, 2017, was 87.2%.

The value of the derivatives on February 17, 2017, amounted to CHF 5.3 million and at period end to CHF 2.8 million; the change in the fair value was recognized in financial income (TCHF 2,540).

## Sensitivity analysis:

	Increase/decrease in volatility assumption	Effect on result before taxes in CHF thousands
Change in volatility	+5%	175
	-5%	-181

**Changes in liabilities arising from financing activities**

	In CHF thousands	
	Convertible bonds	Derivative financial instruments
<b>January 1, 2017</b>	<b>0</b>	<b>0</b>
Proceeds from convertible bonds	60,000	0
Transaction costs relating to convertible bonds	-2,731	0
<b>Cash flows</b>	<b>57,269</b>	<b>0</b>
Non-cash changes		
Initial recognition derivative financial instruments	-5,332	5,332
Change in fair value of derivative financial instruments	0	-2,540
Effective interest/amortized cost calculation	1,174	0
<b>December 31, 2017</b>	<b>53,111</b>	<b>2,792</b>

## 13 Deferred Taxes

### Net deferred taxes recorded

	In CHF thousands	2017	2016
Temporary differences on inventory		1,242	1,067
Temporary difference on accruals		0	39
<b>Deferred tax assets recognized</b>		<b>1,242</b>	<b>1,106</b>
<hr/>			
Temporary differences on intangible assets		1,905	2,154
Temporary differences on intercompany loans		13,449	13,449
Temporary differences on convertible bonds		321	0
Tax loss carryforwards		-15,675	-15,603
<b>Deferred tax liabilities recognized</b>		<b>0</b>	<b>0</b>
<hr/>			
Tax loss carryforwards		339,492	301,667
Of which recorded		-197,008	-195,583
<b>Of which unrecorded</b>		<b>142,484</b>	<b>106,084</b>
<hr/>			
Expiring in			
1 year		22,671	5,832
2 years		30,569	22,671
3 years		4,223	27,366
4 years		0	4,223
5 years		0	0
More than 5 years		54,552	17,942
Without expiration		30,469	28,050
<b>Total unrecorded tax loss carryforwards</b>		<b>142,484</b>	<b>106,084</b>

Due to the uncertainty surrounding the future results of operations and the uncertainty as to whether Santhera can use the loss carryforwards for tax purposes, deferred tax assets on tax loss carryforwards were only considered to the extent that they offset taxable temporary differences within the same taxable entity. As there are no temporary differences associated with investments in subsidiaries, no deferred tax liability has to be recognized. No deferred tax assets are calculated on temporary differences related to pension obligations from IAS 19 (TCHF 8,375 at December 31, 2017, and TCHF 6,183 at December 31, 2016, respectively).

## 14 Trade and Other Payables

	In CHF thousands	2017	2016
Trade payables		3,585	3,574
Other payables (nonfinancial)		1,149	884
<b>Total at December 31</b>		<b>4,734</b>	<b>4,458</b>

All positions are noninterest-bearing and usually settled within 30 to 60 days.

## 15 Accrued Expenses

	In CHF thousands	2017	2016
Development programs		1,547	749
Liabilities to employees		3,429	2,013
Accruals for pricing and reimbursement		839	1,107
Accrued marketing and sales expenses		469	953
Accruals for audit, consulting and other		688	696
Accruals for interest expenses		1,108	0
Accruals for income taxes		432	282
<b>Total at December 31</b>		<b>8,512</b>	<b>5,800</b>

## 16 Commitments and Contingent Liabilities

### Commitments

#### *Commitments for operating lease (noncancelable)*

	In CHF thousands	2017	2016
Within 1 year		1,176	734
After 1 year through to 5 years		1,177	804
After 5 years		34	0
<b>Total at December 31</b>		<b>2,387</b>	<b>1,538</b>

### Contingent liabilities

#### *Collaboration and license agreement with Takeda*

In September 2013, Santhera announced an agreement with Takeda Pharmaceutical Company Ltd, Osaka, Japan (**Takeda**) to license back all previously granted rights in DMD and Friedreich's ataxia (**FA**) in order to increase its strategic flexibility. In return, Takeda is eligible to obtain a percentage from future licensing and/or sales income generated by Santhera in DMD of up to EUR 7.0 million. In addition, Santhera has obtained the right to cross-reference Takeda's idebenone data for regulatory use in any indication and in any territory. If Santhera makes use of such cross-reference right, Takeda is eligible

to obtain a percentage from future licensing and/or sales income generated by Santhera in such indications of up to EUR 3.0 million. Lastly, both companies agreed to terminate a similar agreement for FA signed in 2005 and Santhera's contingent liability of EUR 1.0 million payable to Takeda has been waived. Takeda is eligible to receive up to EUR 1.0 million as a percentage from future income generated by Santhera to offset this waiver.

*Agreement with the University of Leuven*

In March 2005, Santhera entered into an agreement with Katholieke Universiteit Leuven, Leuven, Belgium (**KU Leuven**), under which KU Leuven assigned to Santhera its patents and patent applications relating to the use of idebenone to treat various forms of muscular-dystrophy-related disorders, particularly DMD. Based on this agreement, Santhera has filed patent applications in major territories covering the use of idebenone for the treatment of DMD.

KU Leuven is entitled to a success fee of up to EUR 0.4 million if and when Santhera commercializes any product in a major market, which includes the EU, the US or Japan and certain countries within the EU. In addition, in the event Santhera commercializes the product itself, KU Leuven is entitled to receive 5% royalties on net sales. In the event Santhera grants commercialization rights to a third party, KU Leuven will receive 15% of all the consideration received by Santhera from such third party.

*License agreement with Novartis*

On June 30, 2007, Santhera entered into an agreement with Novartis Pharma AG, Basel, Switzerland (**Novartis**), under which it inlicensed omigapil. Santhera develops omigapil for the treatment of congenital muscular dystrophy (**CMD**). Additional payments will be due to Novartis a) upon start of a pivotal clinical trial, b) upon regulatory approval in a major market country, and c) after reaching certain commercialization milestones. Santhera will also have to pay royalties to Novartis calculated on net sales.

*Agreement with the National Institutes of Health*

In June 2013, Santhera has obtained an exclusive license from the National Institutes of Health, Bethesda/Maryland, US (**NIH**), to its rights on a patent granted in the US for the use of idebenone for the treatment of primary progressive multiple sclerosis (**PPMS**). Under the terms of the agreement, Santhera would have to make certain milestone payments to the NIH not exceeding USD 1.4 million in total. Furthermore, the NIH is eligible to a royalty fee of 3% on net sales and 15% of considerations received in case Santhera sublicenses the program.

*Contracts for clinical development and other*

As part of its ordinary course of business, Santhera has entered into several contracts for e.g. clinical or technical development services. Commitments are within current market prices and can be terminated at the Company's discretion.

**Contingent liabilities summary**

Santhera believes that the disclosures above and accruals (see note 15 "*Accrued Expenses*") are adequate based upon currently available information. However, given the inherent difficulties in estimating liabilities relating to clinical development, regulatory, tax, possible litigation and certain other matters due to uncertainty concerning both the amount and timing of future expenditures, additional costs may be incurred materially beyond the amounts accrued.

## 17 Equity Rights Plans

Santhera has established equity rights plans to align the long-term interests of the members of the Board, the Executive Management and employees. Rights granted under these plans are equity-settled.

### 17.1 Stock Option Plans

#### Executive Incentive Plan (EIP)

In November 2006, under the EIP, the members of the Executive Management were granted stock options to acquire 101,065 Shares, as a management incentive. Each of these stock options entitled its holder to purchase one Share at an exercise price of CHF 1. The vesting period of the options was one year. At the end of the option term, i.e. after a period of ten years as from the grant date, all unexercised stock options will expire without value. The EIP is administered under the responsibility of the Board. No further grants can be made under the EIP.

#### *Options outstanding, exercised or forfeited under the EIP*

Number of options Plan	2017			2016				
	Exercised	For- feited	Expired	Out- standing	Exercised	For- feited	Expired	Out- standing
EIP	0	0	0	0	1,210	0	0	0

#### Employee Stock Option Plans

The Company adopted the ESOP 2004, ESOP 2008, ESOP 2010 and ESOP 2015 (collectively the **ESOP**) to provide incentives to the Executive Management, employees and consultants helping to ensure their commitment to Santhera over the long-term. Option grants were made periodically at the discretion of the Board or as contractually agreed with employees. The ESOP contain customary provisions in respect of the adjustment or cancellation of stock options upon termination of employment, retirement, death, disability and certain corporate transactions. All stock option plans are administered under the responsibility of the Board. Each stock option entitles its holder to purchase one Share of the Company at an exercise price defined to be either a) equal to the volume-weighted average share price in the three preceding months for Swiss employees, or b) the closing share price on the SIX Swiss Exchange (**SIX**) at each grant date. In general, 50% of the stock options vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. At the end of the option term, i.e. after a period of 10 years as from the grant date, unexercised stock options expire without value. Subject to the provisions of the ESOP 2004, vested stock options of employees leaving the Company in good faith do not lapse. Under the ESOP 2008 and ESOP 2010 vested stock options of employees leaving the Company in good faith expire six months after the termination date of the employment. Under the ESOP 2015 vested stock options of employees leaving the Company in good faith do not expire. Unvested stock options of employees leaving the Company are forfeited under all stock option plans. No further grants can be made under the ESOP.

*Options outstanding, exercised, forfeited or expired under ESOPs*

Number of options						2017
	At January 1	Exercised	Granted	Forfeited	Expired	At December 31
ESOP 2004	753	0	0	0	-753	0
ESOP 2010	38,249	-8,698	0	0	0	29,551
ESOP 2015	260,801	0	0	-15,472	0	245,329
<b>Total</b>	<b>299,803</b>	<b>-8,698</b>	<b>0</b>	<b>-15,472</b>	<b>-753</b>	<b>274,880</b>

Number of options						2016
	At January 1	Exercised	Granted	Forfeited	Expired	At December 31
ESOP 2004	26,091	-4,825	0	0	-20,513	753
ESOP 2008	1,500	-1,500	0	0	0	0
ESOP 2010	47,773	-9,524	0	0	0	38,249
ESOP 2015	140,260	0	135,830	-15,289	0	260,801
<b>Total</b>	<b>215,624</b>	<b>-15,849</b>	<b>135,830</b>	<b>-15,289</b>	<b>-20,513</b>	<b>299,803</b>

**Board Stock Option Plans**

The Company adopted the BSOP 2011 and BSOP 2015 (collectively the **BSOP**) to provide incentives to members of the Board. The BSOPs contain the same customary provisions as under the ESOP described above. Each stock option entitles its holder to purchase one Share of the Company at an exercise price defined to be either a) equal to the volume-weighted average share price in the three preceding months, or b) the closing share price on the SIX at each grant date. In general, 50% of the stock options vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. At the end of the option term, i.e. after a period of 10 years as from the grant date, unexercised stock options expire without value. Under the BSOP 2011 vested stock options of Board members leaving the Board in good faith expire six months after the termination date of them being a member of the Board while unvested stock options of Board members leaving the Board in good faith are forfeited. Under the BSOP 2015 vested and unvested stock options of Board members leaving the Board in good faith do not expire. No further grants can be made under the BSOP.

*Options outstanding, exercised, forfeited or expired under BSOPs*

Number of options						<b>2017</b>
	At January 1	Exercised	Granted	Forfeited	Expired	At December 31
BSOP 2015	13,562	0	0	0	0	13,562
<b>Total</b>	<b>13,562</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>13,562</b>

Number of options						<b>2016</b>
	At January 1	Exercised	Granted	Forfeited	Expired	At December 31
BSOP 2015	7,000	0	6,562	0	0	13,562
<b>Total</b>	<b>7,000</b>	<b>0</b>	<b>6,562</b>	<b>0</b>	<b>0</b>	<b>13,562</b>

Since July 1, 2016, no more stock options are available for future grants under the ESOP 2015 and/or the BSOP 2015. Stock option plans were replaced by Share Appreciation Rights (**SAR**), see note 17.2 *"Share Appreciation Rights Plans"*.

**Fair value calculations for stock options granted**

The fair value of stock options is determined at each grant date by using the Hull-White pricing model. The calculation of the option value was performed by applying the following parameters:

	<b>2016</b>
Market price of stock	CHF 37.05 to 91.25
Exercise prices	CHF 69.30 to 89.45
Weighted average fair value of options granted	CHF 24.18
Expected volatility <sup>1</sup>	38% to 39%
CHF risk-free interest rate	0.0% p.a.
Option term <sup>2</sup>	10 years
Expected dividend yield	0%

<sup>1</sup> The expected volatility was determined on the basis of selected biotech companies.

<sup>2</sup> After expiration of the vesting period, the stock options become American-style options and may be exercised any time until the end of the option term. The option-pricing model takes into consideration certain assumptions about potential early exercises.

**Number of stock options outstanding and exercisable**

	Number of options	2017	2016
<b>Outstanding at January 1</b>		<b>313,365</b>	<b>223,834</b>
Granted		0	142,392
Exercised <sup>1</sup>		-8,698	-17,059
Forfeited		-15,472	-15,289
Expired		-753	-20,513
<b>Outstanding at December 31</b>		<b>288,442</b>	<b>313,365</b>
<b>Exercisable at December 31</b>		<b>102,642</b>	<b>36,327</b>

<sup>1</sup> The average closing share price of options exercised during the reporting period 2017 was CHF 39.05 (2016: CHF 68.12).

The value of stock options granted is recognized as personnel expense over the period Santhera receives services. In 2017, previously granted stock options resulted in personnel expenses of TCHF 2,778 (TCHF 374 related to Development, TCHF 1,525 related to Marketing and sales (M&S) and TCHF 879 to General and administrative (G&A)) and in 2016, such grants resulted in personnel expenses of TCHF 3,311 (TCHF 418 related to Development, TCHF 1,766 related to M&S and TCHF 1,127 to G&A).

**Terms of options outstanding at December 31**

Exercise price range for options (in CHF)	Number outstanding	Weighted average remaining contractual life (years)	2017 Number exercisable	Number outstanding	Weighted average remaining contractual life (years)	2016 Number exercisable
from 3.85 to 4.53	25,001	5.13	25,001	33,699	6.66	33,574
at 22.25	4,550	6.50	3,275	4,550	7.48	2,000
at 69.30	14,800	8.25	0	18,800	9.23	0
from 82.10 to 114.50	244,091	7.73	74,366	256,316	8.61	753
<b>Total</b>	<b>288,442</b>	<b>7.61</b>	<b>102,642</b>	<b>313,365</b>	<b>8.42</b>	<b>36,327</b>

**17.2 Share Appreciation Rights Plans**

Starting with July 1, 2016, Santhera switched from stock option plans to Share Appreciation Rights Plans (SARP). It introduced a Board Share Appreciation Plan 2016 (BSARP 2016) and an Employee Share Appreciation Rights Plan 2016 (ESARP 2016). In 2017 Santhera has introduced a Board Share Appreciation Rights Plan (BSARP 2017) for the members of its Board and an Employee Share Appreciation Rights Plan (ESARP 2017) for the Executive Management, employees and consultants. SAR grants are made periodically at the discretion of the Board or as contractually agreed with employees. The SARP contain customary provisions in respect of the adjustment or cancellation of SARs upon termination of employment, retirement, death, disability and certain corporate transactions. All SARPs are administered under the responsibility of the Board. In general, 50% of the SARs vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. SARP introduced in 2017 (BSARP 2017 and ESARP 2017) foresee vesting of 1/3 of the SAR on the first anniversary; the remaining 2/3 vest by each following quarter end through the second and third year after the grant date

(8 times 1/12 of the SAR granted). At the end of the SAR term, i.e. after a period of 10 years as from the grant date, unexercised SARs expire without value. Upon exercise of one SAR, participants receive the difference between the price of one Share at the time of exercise and the base value ("exercise price" as defined upon grant), in Shares. Subsequently, participants may sell their Shares.

*SAR outstanding, exercised, forfeited or expired under SARP*

Number of SAR						2017
	At January 1	Exercised	Granted	Forfeited	Expired	At December 31
ESARP 2016	56,581	0	63,889	-34,881	0	85,589
BSARP 2017	0	0	15,120	0	0	15,120
ESARP 2017	0	0	271,234	-11,833	0	259,401
<b>Total</b>	<b>56,581</b>	<b>0</b>	<b>350,243</b>	<b>-46,714</b>	<b>0</b>	<b>360,110</b>

Number of SARs						2016
	At January 1	Exercised	Granted	Forfeited	Expired	At December 31
ESARP 2016	0	0	56,581	0	0	56,581
<b>Total</b>	<b>0</b>	<b>0</b>	<b>56,581</b>	<b>0</b>	<b>0</b>	<b>56,581</b>

Fair value calculations for SAR granted

The fair value of SAR is determined at each grant date by using the Hull-White pricing model. The calculation of the SAR value was performed by applying the following parameters:

	2017	2016
Market price of stock	CHF 29.00 to 82.00	CHF 37.05 to 91.25
Exercise prices	CHF 38.60 to 77.80	CHF 51.75 to 76.50
Weighted average fair value of SAR granted	CHF 25.94	CHF 22.12
Expected volatility <sup>1</sup>	38%	38% to 39%
CHF risk-free interest rate	0.0% p.a.	0.0% p.a.
SAR term <sup>2</sup>	10 years	10 years
Expected dividend yield	0%	0%

<sup>1</sup> The expected volatility was determined on the basis of selected biotech companies.

<sup>2</sup> After expiration of the vesting period, the SARs become rights similar to American-style options and may be exercised any time until the end of the SAR term. The SAR pricing model takes into consideration certain assumptions about potential early exercises.

**Number of SAR outstanding and exercisable**

	Number of SAR	2017	2016
<b>Outstanding at January 1</b>		<b>56,581</b>	<b>0</b>
Granted		350,243	56,581
Exercised		0	0
Forfeited		-46,714	0
Expired		0	0
<b>Outstanding at December 31</b>		<b>360,110</b>	<b>56,581</b>
<b>Exercisable at December 31</b>		<b>0</b>	<b>0</b>

The value of SAR granted is recognized as personnel expense over the period Santhera receives services. In 2017, SAR grants resulted in personnel expenses of TCHF 4,517 (TCHF 1,513 related to Development, TCHF 1,513 related to M&S and TCHF 1,491 to G&A) and in 2016, such grants resulted in personnel expenses of TCHF 150 (TCHF 15 related to Development, TCHF 135 related to M&S and TCHF 0 to G&A).

Santhera plans to allocate up to 444,164 SAR in the first quarter of 2018 (in the first quarter 2017, it was planned to allocate up to 198,162 SAR). These SAR form part of the long-term incentive (LTI) award to employees for the year ended December 31, 2017. Although these SAR were not legally granted in 2017, Executive Management considers it appropriate to recognize expenses in 2017 as employees have been rendering services in 2017 in expectation of the annual LTI allocation. Personnel expenses in 2017 for this amounted to TCHF 2,392 (TCHF 825 related to Development, TCHF 655 related to M&S and TCHF 912 related to G&A) based on an estimate of fair value (in 2016 personnel expenses for this amounted to TCHF 1,222 (TCHF 332 related to Development, TCHF 569 related to M&S and TCHF 321 related to G&A)). The allocation of these SAR is conditional for the Executive Management and becomes unconditional once the compensation is approved on the occasion of the AGM, to be held on April 12, 2018. After the AGM the grant date fair value of the SAR will be determined and the cumulative expense adjusted.

**Terms of SAR outstanding at December 31**

Exercise price range for SAR (in CHF)	Number outstanding	Weighted average re-maining contractual life (years)	2017 Number exercisable	Number outstanding	Weighted average re-maining contractual life (years)	2016 Number exercisable
at 38.70	33,257	9.76	0	n/a	n/a	n/a
from 51.75 to 76.50	283,435	9.01	0	56,581	9.71	0
from 76.50 to 77.80	43,418	9.08	0	n/a	n/a	n/a
<b>Total</b>	<b>360,110</b>	<b>9.04</b>	<b>0</b>	<b>56,581</b>	<b>9.71</b>	<b>0</b>

## 18 Segment and Geographic Information

### Segment information

Santhera operates in one operating segment, the development and commercialization of specialty niche products for the treatment of neuro-ophthalmological, neuromuscular and pulmonary diseases. The Board, the Executive Management and senior managers, being the CODM, assess the reporting data and allocate resources as one segment on a consolidated level according to the operating expenses by function. Santhera generates revenue from sales of Raxone for the treatment of LHON. Geographic revenue information is based on location of the customer.

### Geographic information

#### *Net sales*

	In CHF thousands	2017	2016
EU		22,859	19,002
Rest of the world		84	31
<b>Total</b>		<b>22,943</b>	<b>19,033</b>

In 2017, net sales amounted to CHF 22.9 million (2016: CHF 19.0 million). Raxone was sold in 20 European countries, with the majority of sales reached in France and Germany (2016: 15 European countries).

#### *Noncurrent assets (excluding financial instruments, restricted cash and deferred taxes)*

	In CHF thousands	2017	2016
Switzerland		25,451	26,966
EU		171	100
North America		95	0
<b>Total</b>		<b>25,717</b>	<b>27,066</b>

## 19 Other Operating Income

This position consists primarily of reimbursements from scientific programs.

## 20 Operating Expenses by Nature

	In CHF thousands	2017	2016
External Development expenses		-14,762	-12,119
Patent and license expenses		-381	-280
Marketing expenses		-13,018	-10,121
Employee expenses		-35,488	-21,403
<i>Of which non-cash-relevant expenses for equity rights plans</i>		<i>-9,687</i>	<i>-4,683</i>
Other administrative expenses		-4,727	-3,796
Depreciation and amortization		-343	-225
Lease expenses		-780	-587
Other operating expenses		-64	-107
<b>Total operating expenses</b>		<b>-69,563</b>	<b>-48,638</b>

## 21 Employee Expenses and Benefits

### Employee expenses

	In CHF thousands	2017	2016
Wages and salaries		-18,372	-12,397
Social security and other personnel-related expenses <sup>1</sup>		-7,429	-4,323
<i>Of which non-cash-relevant adjustments of pension fund</i>		<i>-2,021</i>	<i>-450</i>
Expenses for equity rights plans		-9,687	-4,683
<b>Total employee costs</b>		<b>-35,488</b>	<b>-21,403</b>

<b>Average number of full-time equivalents<sup>2</sup></b>	<b>92.9</b>	<b>65.1</b>
<b>Full-time equivalents at year-end</b>	<b>106.2</b>	<b>74.4</b>
<b>Total headcount at year-end</b>	<b>112</b>	<b>80</b>

<sup>1</sup> Thereof TCHF 306 were expensed for defined contribution plans in North America and some EU countries (2016: TCHF 124).

<sup>2</sup> For the calculation of full-time equivalents, only employees with part-time and full-time permanent working contracts are taken into consideration.

### Pension plan

In accordance with the Swiss pension fund law "Federal Act on Occupational Old Age, Survivors' and Invalidity Pension Provision" (OPA), all employees of Santhera Pharmaceuticals Holding AG, Liestal, and Santhera Pharmaceuticals (Schweiz) AG, Pratteln, both in Switzerland, have to be affiliated with a collective independent pension fund. These funds provide for retirement benefits, as well as risk benefits (death and disability). The plans qualify as defined benefit plans under IAS 19 and the assets cannot revert to the employer. Contributions to the plans are such that the employee contributes 40% and the employer the rest. Contributions are computed as percentage of the salary, depending on age. In order to manage these risks, Santhera had an agreement with AXA Foundation for occupational benefits (AXA

**foundation)** during 2017. As of January 1, 2018, Santhera has entered into an agreement with PKG Pensionskasse (PKG) and changed its pension fund provider, effective as of this date. The AXA foundation and PKG are responsible for the governance of the plan; their boards are composed of an equal number of representatives from the employers and employees chosen from all affiliated companies. AXA foundation and PKG have set up investment guidelines, defining in particular the strategic allocation with margins. AXA foundation has reinsured its risks (investment risk, mortality risk, etc.) with AXA Life Ltd, Winterthur, Switzerland (AXA), whereas PKG has only insured the risks disability and death before retirement with PKRück AG, Vaduz, Fürstentum Liechtenstein. The accumulated savings capital is allocated to each insured individual and consists of annual contributions, savings credits and interest credits. In certain situations, additional payments or increased periodic contributions by the employer may become due based on the pension plans funded status as measured under Swiss pension rules (OPA).

An independent actuary has performed the respective calculations as required by IAS 19:

*Changes in defined benefit obligations*

	In CHF thousands	2017	2016
<b>Present value of obligation, January 1</b>		<b>21,279</b>	<b>15,797</b>
Current employer service cost		1,614	1,038
Past service expense <sup>1</sup>		1,240	0
Interest cost		138	142
Employee contributions		514	382
Benefits paid / transfer payments		-620	2,259
Insurance premiums		-266	-192
Remeasurements <sup>2</sup>		253	1,853
<b>Present value of obligation, December 31</b>		<b>24,152</b>	<b>21,279</b>

<sup>1</sup> Increase of obligation due to increase of the conversion rates for the over-mandatory part of the retirement capital under the new PKG plan rules (based on the agreements signed in November 2017).

<sup>2</sup> Details of remeasurements:

	In CHF thousands	2017	2016
Effect of changes in demographic assumptions		0	-435
Actuarial gain/loss due to changes in financial assumptions		-400	599
Actuarial gain/loss due to experience adjustments		653	1,689
<b>Subtotal gain/loss</b>		<b>253</b>	<b>1,853</b>
Return/loss on plan assets (excluding interest income)		-82	-77
<b>Total remeasurements in other comprehensive income gain/loss</b>		<b>171</b>	<b>1,776</b>

*Changes in plan assets*

	In CHF thousands	2017	2016
<b>Fair value of assets, January 1</b>		<b>15,096</b>	<b>11,840</b>
Interest income on assets		101	110
Employer contributions		870	620
Employee contributions		514	382
Benefits paid / transfer payments		-620	2,259
Insurance premiums		-266	-192
Remeasurements (return/loss on plan assets (excluding interest income))		82	77
<b>Fair value of assets, December 31</b>		<b>15,777</b>	<b>15,096</b>

*Net defined benefit asset/obligation*

	In CHF thousands	2017	2016
Present value of obligation, December 31		24,152	21,279
Fair value of assets, December 31		15,777	15,096
<b>Net defined asset/obligation</b>		<b>-8,375</b>	<b>-6,183</b>

*Asset breakdown*

	In CHF thousands		2017		2016	
	Quoted market price	Not quoted market price	Quoted market price	Not quoted market price	Quoted market price	Not quoted market price
Insurance contract	0	15,777	0	15,096	0	15,096
<b>Total value of assets</b>	<b>0</b>	<b>15,777</b>	<b>0</b>	<b>15,096</b>	<b>0</b>	<b>15,096</b>

An asset breakdown is not available. The assets of Santhera's defined benefit plan have no quoted market price since AXA fully insures them as an insurance contract.

*The weighted average assumptions to determine benefit obligations and defined benefit cost were as follows:*

	In %	2017	2016
Discount rate		0.70	0.65
Expected future salary increases		1.50	1.50

*Sensitivity analysis for 2017:*

In CHF thousands	Defined benefit obligation		Gross (net) service cost	
	Increase assumption	Decrease assumption	Increase assumption	Decrease assumption
Discount rate +/-0.25%	-1,027	1,105	-129	137
Salary increase +0.25%	156	-	0	-
Life expectancy +1 year	448	-	35	-

*Sensitivity analysis for 2016:*

In CHF thousands	Defined benefit obligation		Gross (net) service cost	
	Increase as- sumption	Decrease assumption	Increase assumption	Decrease assumption
Discount rate +/-0.25%	-896	961	-117	125
Salary increase +0.25%	130	-	-1	-
Life expectancy +1 year	431	-	32	-

*Mortality rate:*

Life expectancy at age 65	<b>2017</b>	<b>2016</b>
Male	22.5	22.4
Female	24.5	24.4

The expected employer contributions for fiscal year 2018 amount to approximately TCHF 936 (2016: TCHF 876). Benefit obligations of pensioners amounted to TCHF 3,103 at December 31, 2017 (2016: none). The duration of the plan liabilities calculated is 20.3 years as of December 31, 2017 (2016: 21.6 years).

**22 Financial Income/Expenses****Financial income**

	In CHF thousands	2017	2016
Interests on cash and cash equivalents		5	5
Change in fair value of financial derivative instruments		2,540	0
Income from financial assets		267	0
Realized and unrealized foreign exchange gains		1,322	923
<b>Total</b>		<b>4,134</b>	<b>928</b>

**Financial expenses**

	In CHF thousands	2017	2016
Interest expenses		-3,843	-15
Expenses from financial assets		-197	0
Realized and unrealized foreign exchange losses		-915	-980
<b>Total</b>		<b>-4,955</b>	<b>-995</b>

**23 Income Taxes**

	In CHF thousands	2017	2016
Current income tax income/expense		-390	-266
Deferred tax income/expense		133	-1,955
<b>Total</b>		<b>-257</b>	<b>-2,221</b>

The following is a theoretical reconciliation of tax expense and the accounting profit multiplied by expected income tax rate of principal:

	In CHF thousands	2017	2016
Result before taxes		-51,275	-33,194
Tax expense/income at expected group tax rate of 9.3%		4,769	3,087
Effect of tax rate difference group versus local		-422	-724
Effect of nondeductible expenses		-784	-372
Prior year DTA (deferred tax assets) decrease		-289	-249
Utilization of previously unrecognized tax losses		24	20
Recognition of previously unrecognized DTL (deferred tax liabilities)		0	-13,449
Recognition of DTA on previously unrecognized tax losses		0	13,449
Unrecognized deferred taxes		-3,555	-3,983
<b>Effective tax income/expense</b>		<b>-257</b>	<b>-2,221</b>

According to currently applicable Swiss tax law, the period to offset tax loss carryforwards against taxable profit is limited to seven years. According to currently applicable German tax law, tax loss carryforwards can, besides other conditions, be offset against taxable profit for an unlimited period but only to an amount of EUR 1.0 million and in addition for 60% of further amounts beyond this threshold per annum.

## 24 Earnings/Loss per Share

Basic earnings/loss per share is calculated by dividing the net profit/net loss attributable to equity holders by the weighted average number of Shares issued and outstanding during the reporting period, excluding Shares held as treasury shares (purchased at market).

	<b>2017</b>	<b>2016</b>
Net result attributable to shareholders (in CHF)	-51,531,770	-35,414,845
Weighted average number of shares issued and outstanding	6,269,813	6,273,460
<b>Basic and diluted net result per share (in CHF)</b>	<b>-8.22</b>	<b>-5.65</b>

For the years ended December 31, 2017 and 2016, basic and diluted net result per share is based on the weighted average number of Shares issued and outstanding and excludes Shares to be issued upon the future exercise of equity rights and upon conversion of the convertible bonds, as they would be anti-dilutive. In case Santhera shows a profit in the future, equity rights and convertible bonds upon conversion may have a dilutive effect on the net profit per Share and will need to be considered for the purpose of this calculation.

## 25 Related Party Transactions

### Board and Executive Management compensation

#### *Total compensation of Board and Executive Management*

	In CHF thousands	<b>2017</b>	<b>2016</b>
Compensation, wages and salaries		3,344	2,546
Post-employment benefits (pension fund contributions)		272	221
Share-based payment expenses (fair value according to IFRS 2)		3,826	1,508
<b>Total</b>		<b>7,442</b>	<b>4,275</b>

#### *Transactions with members of the Board and Executive Management*

There are no loans outstanding or guarantee commitments granted to members of the Board and Executive Management.

In 2017, no stock options were exercised by members of the Board (2016: no stock options exercised). During 2017, 5,000 stock options were exercised by the Executive Management (2016: 3,999 stock options exercised).

## 26 Risk Management Objectives and Policies

Santhera Pharmaceuticals Holding AG maintains a Group-wide corporate risk management system consisting of the areas corporate governance, financial internal controls and quality control / quality assurance.

On a regular basis, operational corporate risks are identified and their likelihood and impact assessed (gross risks). By defining and undertaking appropriate measures, these risks are managed accordingly to either reduce or avoid such risk (net risk). The results of this process are discussed at Board meetings.

Those risks as identified within the area of accounting and financial reporting as well as related control processes are further covered by the Company's Group-wide internal control system.

Santhera conducts development activities primarily in Switzerland, the EU and the US and is exposed to a variety of financial risks, such as, but not limited to, foreign exchange rate risk, credit risk, liquidity risk, cash flow and interest rate risk. Part of Santhera's overall risk management focuses on financial risks and the unpredictability of financial markets seeking to minimize potential adverse effects on the financial performance of the Group. Special guidelines and policies approved by the Board exist for overall risk management, financial internal controls and treasury management and are monitored by the Executive Management and the Board on a regular basis. The risk of foreign exchange rate fluctuations on the expenses can partly be managed by entering into foreign exchange derivative contracts. In accordance with the relevant treasury guidelines, Santhera only concludes contracts with selected high-quality financial institutions of good reputation and is not allowed to engage in speculative transactions. In addition, Santhera's treasury guidelines limit the Company to engage in money market deposits or similar instruments with a maturity beyond 6 months.

### Foreign exchange rate risk

Santhera holds cash amounts in five major currencies CHF, EUR, USD, GBP and CAD to cover the majority of future expected expenses. In addition, in order to reduce its foreign exchange rate exposure, Santhera occasionally enters into derivative currency contracts (forwards, options, structured derivatives) to hedge against additional major foreign currency exchange rate fluctuations. Evaluations based on market values are performed regularly. Any fair value changes of such currency positions are recorded accordingly in the income statement. Santhera's primary exposure to financial risk is due to fluctuation of exchange rates between CHF, EUR, USD, GBP and CAD. No derivative currency contracts are outstanding as of December 31, 2017 and 2016.

The following table demonstrates the sensitivity to a reasonable possible change in the EUR exchange rate, with all other variables held constant, of the Group's result before taxes. There is no impact on the Group's equity.

	Increase/decrease foreign currency rate	Effect on result before taxes in CHF thousands
<b>EUR positions</b>		
<b>2017</b>	+5%	+411
	-5%	-411
<b>2016</b>	+5%	+187
	-5%	-187

### **Interest rate risk**

Santhera earns interest income on cash and cash equivalents and its profit and loss may be influenced by changes in market interest rates. Santhera holds its cash on deposit/current accounts or invests cash through deposits in line with its treasury guidelines to follow its financial needs over time.

The following calculation demonstrates the sensitivity to a reasonable change in interest rates, with all other variables held constant, of the Group's result before taxes. There is no impact on the Group's equity.

As of the end of 2017, variances of  $\pm 50$  basis points were calculated, resulting in fluctuations of  $\pm$  TCHF 263 before tax (end of 2016:  $\pm 50$  basis points resulting in fluctuations of  $\pm$  TCHF 249 before tax).

Additionally, Santhera's interest rate risk arises from long-term debt. Debt issued at fixed rates exposes the Group to fair value interest rate risk.

### **Credit risk**

Santhera has a certain concentration of credit risk. Short-term investments are invested as cash on deposit or in low-risk money market funds. No investment or contract with any single counterparty, except cash on deposit subject to the criteria above, comprises more than 30% of cash and cash equivalents at the date of investment.

Santhera has policies in place to ensure that sales of products or entered partnerships are made to or entered with customers or partners with an appropriate credit history and a commitment to ethical business practices. The maximum credit risk exposure is limited to the carrying amount of its financial assets including derivatives.

### **Liquidity risk**

Prudent liquidity risk management implies maintaining sufficient cash and cash equivalents. Currently, the Company is financed through equity and convertible bonds (see note 12 "*Financial Assets and Liabilities*"). Santhera's treasury calculates on a rolling basis the needs for aligning the current expenses against the need for optimized financial investments.

*Contractual undiscounted cash flows*

	Year ended December 31, 2017					Total	Book value
	On demand	Less than 3 months	3 to 12 months	1 to 5 years	In CHF thousands		
Convertible bonds	0	1,500	1,500	70,500	73,500	53,111	
Trade payables	0	3,585	0	0	3,585	3,585	
Accrued expenses	0	5,083	0	0	5,083	5,083	
<b>Total</b>	<b>0</b>	<b>10,168</b>	<b>1,500</b>	<b>70,500</b>	<b>82,168</b>	<b>61,779</b>	

	Year ended December 31, 2016					Total	Book value
	On demand	Less than 3 months	3 to 12 months	1 to 5 years	In CHF thousands		
Trade payables	0	3,574	0	0	3,574	3,574	
Accrued expenses	0	3,787	0	0	3,787	3,787	
<b>Total</b>	<b>0</b>	<b>7,361</b>	<b>0</b>	<b>0</b>	<b>7,361</b>	<b>7,361</b>	

**Categories of financial instruments**

Year ended December 31, 2017 In CHF thousands	Book value	Loans and receivables	Other liabilities at amortized cost	At fair value through profit or loss	
<b>Assets</b>					
Financial assets long-term	713	713	0	0	0
Restricted cash long-term	4,500	4,500	0	0	0
Trade receivables	4,194	4,194	0	0	0
Other receivables	149	149	0	0	0
Financial assets short-term	13,011	13,011	0	0	0
Restricted cash short-term	3,000	3,000	0	0	0
Cash and cash equivalents	45,195	45,195	0	0	0
<b>Total</b>	<b>70,762</b>	<b>70,762</b>	<b>0</b>	<b>0</b>	<b>0</b>
<b>Liabilities</b>					
Convertible bonds	53,111	0	53,111	0	0
Derivative financial instruments	2,792	0	0	2,792	0
Trade payables	3,585	0	3,585	0	0
Accrued expenses	5,083	0	5,083	0	0
<b>Total</b>	<b>64,571</b>	<b>0</b>	<b>61'779</b>	<b>2,792</b>	<b>0</b>

Year ended December 31, 2016 In CHF thousands	Book value	Loans and receivables	Other liabilities at amortized cost	At fair value through profit or loss
<b>Assets</b>				
Financial assets long-term	270	270	0	0
Trade receivables	3,412	3,412	0	0
Other receivables	55	55	0	0
Cash and cash equivalents	49,815	49,815	0	0
<b>Total</b>	<b>53,552</b>	<b>53,552</b>	<b>0</b>	<b>0</b>
<b>Liabilities</b>				
Trade payables	3,574	0	3,574	0
Accrued expenses	3,787	0	3,787	0
<b>Total</b>	<b>7,361</b>	<b>0</b>	<b>7,361</b>	<b>0</b>

### Capital management

The first priority of Santhera's capital management is to provide adequate cash funds to ensure the financing of successful development and marketing activities so that future profits can be generated by gaining marketing authorization approvals for pharmaceutical products. As a company with currently only one marketed product, the capital management continues to be focused on the cash and cash equivalents position and is governed by specific Group treasury guidelines.

The funds raised in various private financing rounds, private placements in 2008, 2014 and 2015, SEDA (Standby Equity Distribution Agreement), the sale of Shares by an independent broker, convertible bonds as well as funds generated through product sales and revenue from licensing enabled the Group to be adequately financed.

Minor changes in goals and policies of the treasury management have been made in 2017, such as e.g. the extension from 20% to 30% for short-term investments with one counterparty or the possibility of physical cash deposits (there were no changes in 2016).

## 27 Events after the Reporting Date

On January 26, 2018, Santhera announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) maintained its negative opinion on the Type II extension application for idebenone in Duchenne muscular dystrophy (DMD) following a re-examination procedure. The CHMP concluded that an approval for idebenone in DMD, applied as a Type II variation of the existing marketing authorization, cannot be granted at the present time based on the current existing evidence. Although the positive outcome of the Phase III DELOS trial was acknowledged, the CHMP has invited Santhera to present additional data to further link the observed treatment effects on respiratory function outcomes to patient benefit. Management has considered the impact on the Group's ability to continue as a going concern and potential indicators of impairment on the financial statements as of December 31, 2017.

On February 15, 2018, Santhera announced that it has entered into a license agreement with Polyphor Ltd., Allschwil, Switzerland, for POL6014, a clinical stage selective inhibitor of human neutrophil elastase with the potential to treat cystic fibrosis (**CF**) and other neutrophilic pulmonary diseases such as non-cystic fibrosis bronchiectasis (**NCFB**), alpha-1 antitrypsin deficiency (**AATD**) and primary ciliary dyskinesia (**PCD**).

Santhera will assume the global development, regulatory filings and commercialization of POL6014. The development program has been advanced with financial support by the Cystic Fibrosis Foundation Therapeutics Inc. (**CFFT**), USA, to Polyphor. With POL6014, Santhera is expanding its product pipeline in pulmonary diseases where the Company is already developing its lead product idebenone for respiratory complication in Duchenne muscular dystrophy.

Under the agreement, Santhera obtains the worldwide, exclusive rights to develop and commercialize POL6014, an innovative macrocycle elastase inhibitor, and analogs for an initial payment of CHF 6.5 million, payable in Santhera shares at an agreed valuation of CHF 27.2053 per share and additional cash payments of up to CHF 121 million contingent to future development, regulatory and particularly sales milestones. In addition, Polyphor is entitled to tiered royalty payments from Santhera's future net sales of POL6014 and to undisclosed milestone payments and royalties provided that Santhera advances the development and market entry of POL6014 in other pulmonary diseases. Santhera issued the 238,924 shares (3.8% of its currently issued shares) required for the initial payment to Polyphor out of its existing authorized share capital.



Ernst & Young Ltd  
Aeschengraben 9  
P.O. Box  
CH-4002 Basle

Phone +41 58 286 86 86  
Fax +41 58 286 86 00  
www.ey.com/ch

To the General Meeting of  
Santhera Pharmaceuticals Holding Ltd, Liestal

Basle, March 19, 2018

## Statutory auditor's report on the audit of the consolidated financial statements



### Opinion

We have audited the consolidated financial statements of Santhera Pharmaceuticals Holding Ltd and its subsidiaries (the Group), which comprise the consolidated balance sheet as at December 31, 2017, and the consolidated income statement, consolidated statement of comprehensive income, consolidated cash flow statement and consolidated statement of changes in equity for the year then ended, and notes to the consolidated financial statements, including a summary of significant accounting policies.

In our opinion, the consolidated financial statements (pages 16 to 57) give a true and fair view of the consolidated financial position of the Group as at December 31, 2017, and its consolidated financial performance and its consolidated cash flows for the year then ended in accordance with International Financial Reporting Standards (IFRS) and comply with Swiss law.



### Basis for opinion

We conducted our audit in accordance with Swiss law, International Standards on Auditing (ISAs) and Swiss Auditing Standards. Our responsibilities under those provisions and standards are further described in the *Auditor's Responsibilities for the Audit of the Consolidated Financial Statements* section of our report.

We are independent of the Group in accordance with the provisions of Swiss law and the requirements of the Swiss audit profession, as well as the IESBA Code of Ethics for Professional Accountants, and we have fulfilled our other ethical responsibilities in accordance with these requirements.

We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our opinion.



### Key audit matters

Key audit matters are those matters that, in our professional judgment, were of most significance in our audit of the consolidated financial statements of the current period. These matters were addressed in the context of our audit of the consolidated financial statements as a whole, and in forming our opinion thereon, and we do not provide a separate opinion on these matters. For each matter below, our description of how our audit addressed the matter is provided in that context.



We have fulfilled the responsibilities described in the *Auditor's responsibilities for the audit of the consolidated financial statements* section of our report, including in relation to these matters. Accordingly, our audit included the performance of procedures designed to respond to our assessment of the risks of material misstatement of the consolidated financial statements. The results of our audit procedures, including the procedures performed to address the matters below, provide the basis for our audit opinion on the consolidated financial statements.

### **Management's assessment of the Group's ability to continue as a going concern**

**Areas of focus** Based on the stage of the operations combined with recurring development costs and the level of cash inflows from its operating activities, the Group incurred negative cash flows from operations in the past and it expects to continue to incur such costs and negative cash flows in the near future. Management has prepared various budget scenarios and business plans and has assessed the likelihood and impact that uncertain cash flows could have on the Group's ability to continue as going concern. This assessment was based on the evaluation of the available year-end funds of CHF 45.2 million as well as the budget.

Despite the Group's development into a more sales oriented business, funding from non-revenue related sources is still required in the future and we therefore concluded that the assessment of the ability to continue as a going concern represents a key audit matter for our audit.

**Our audit response** Our audit procedures related to the key audit matter of the assessment of the going concern included the following procedures:

- We gained an understanding of management's budgeting and forecasting process underlying the going concern assessment.
- We discussed the budget and business plans with management, evaluated the development and the assessments made and also considered how uncertain elements were included in the budget and business plans. We evaluated underlying key assumptions such as expected cash inflow from product sales and expected cash outflow from purchases of inventory, research and development expenses and other operating expenses.
- We tested the sensitivity of the revenue estimates and assessed how they compared to historical revenues.
- We further discussed the base case scenario with management and assessed the likelihood of this scenario.
- We performed inquiries of management about current developments and the ability to execute the elements included in the budget and business plans supporting the evaluation of the going concern assumption. We read the Board of Directors meeting minutes that approved the underlying budget to assess potential contrary information. We performed procedures regarding subsequent events to assess the accuracy of the budget as of the date of the auditor's report.

### Accounting for board and employee share appreciation rights

**Areas of focus** The Group operates several equity right plans for its employees and board members. Grants are made periodically at the discretion of the board or as contractually agreed with employees. The fair value of the equity rights and ultimately personnel expenses to be recognized are determined based upon assumptions. In the reporting period 2017 expenses recorded in reference to equity right plans amounted to CHF 9.7 million compared to CHF 4.7 million in the same period in 2016.

*Refer to note 17 to these financial statements disclosures related to equity rights plans.*

**Our audit response** We tested the fair value determination for all grants in the year 2017 and assessed the accuracy of the share-based payment expenses recognized. This included, among others, the following procedures:

- We obtained and read documentation related to new share appreciation right plans established during financial year 2017 to understand the terms under which these rights were granted.
- We inspected new grants on a sample basis and referenced the grants to supporting documentation such as the communication to the employees. Further, we reconciled the number of awards granted to the calculation of the expenses and recalculated the amounts to be recognized over the vesting period.
- We assessed management's assumptions used in the calculation of the expenses by comparing these to market and historical data. Assumptions assessed included forfeiture rates.
- We also considered the adequacy of the disclosures made in relation to share-based payments.



### Other information in the annual report

The Board of Directors is responsible for the other information in the annual report. The other information comprises all information included in the annual report, but does not include the consolidated financial statements, the stand-alone financial statements, the compensation report and our auditor's reports thereon.

Our opinion on the consolidated financial statements does not cover the other information in the annual report and we do not express any form of assurance conclusion thereon.

In connection with our audit of the consolidated financial statements, our responsibility is to read the other information in the annual report and, in doing so, consider whether the other information is materially inconsistent with the consolidated financial statements or our knowledge obtained in the audit, or otherwise appears to be materially misstated. If, based on the work we have performed, we conclude that there is a material misstatement of this other information, we are required to report that fact. We have nothing to report in this regard.



### **Responsibility of the Board of Directors for the consolidated financial statements**

The Board of Directors is responsible for the preparation of the consolidated financial statements that give a true and fair view in accordance with IFRS and the provisions of Swiss law, and for such internal control as the Board of Directors determines is necessary to enable the preparation of consolidated financial statements that are free from material misstatement, whether due to fraud or error.

In preparing the consolidated financial statements, the Board of Directors is responsible for assessing the Group's ability to continue as a going concern, disclosing, as applicable, matters related to going concern and using the going concern basis of accounting unless the Board of Directors either intends to liquidate the Group or to cease operations, or has no realistic alternative but to do so.



### **Auditor's responsibilities for the audit of the consolidated financial statements**

Our objectives are to obtain reasonable assurance about whether the consolidated financial statements as a whole are free from material misstatement, whether due to fraud or error, and to issue an auditor's report that includes our opinion. Reasonable assurance is a high level of assurance, but is not a guarantee that an audit conducted in accordance with Swiss law, ISAs and Swiss Auditing Standards will always detect a material misstatement when it exists. Misstatements can arise from fraud or error and are considered material if, individually or in the aggregate, they could reasonably be expected to influence the economic decisions of users taken on the basis of these consolidated financial statements.

A further description of our responsibilities for the audit of the consolidated financial statements is located at the website of EXPERTsuisse: <http://www.expertsuisse.ch/en/audit-report-for-public-companies>. This description forms part of our auditor's report.



### **Report on other legal and regulatory requirements**

In accordance with article 728a para. 1 item 3 CO and the Swiss Auditing Standard 890, we confirm that an internal control system exists, which has been designed for the preparation of consolidated financial statements according to the instructions of the Board of Directors.

We recommend that the consolidated financial statements submitted to you be approved.

Ernst & Young Ltd

/s/ Frederik Schmachtenberg

Licensed audit expert  
(Auditor in charge)

/s/ Jan Meyer

Licensed audit expert

# Statutory Financial Statements of Santhera Pharmaceuticals Holding AG

## Contents

Balance Sheet .....	63
Income Statement .....	64
Notes to the Statutory Financial Statements .....	65
1 Introduction .....	65
2 Principles .....	65
3 Information on Balance Sheet and Income Statement Items .....	66
4 Other Information .....	69
Proposal of the Board of Directors to the Annual General Meeting .....	74
Report of the Statutory Auditor on the Financial Statements .....	75

## Balance Sheet

	As of December 31, in CHF thousands	Notes	2017	2016
<b>Assets</b>				
Cash and cash equivalents			30,738	43,187
Financial assets short-term			13,011	0
Other receivables from third parties			54	24
Prepaid expenses and accrued income			153	46
Restricted cash short-term			3,000	0
<b>Current assets</b>			<b>46,956</b>	<b>43,257</b>
Loans to shareholdings		3.1	63,293	17,727
Investments in shareholdings		3.2	198	115
Restricted cash long-term			4,500	0
<b>Noncurrent assets</b>			<b>67,991</b>	<b>17,842</b>
<b>Total assets</b>			<b>114,947</b>	<b>61,099</b>
<b>Liabilities and equity</b>				
Trade accounts payable to third parties			311	97
Other accounts payable to third parties			35	56
Accrued expenses			1,538	453
<b>Current liabilities</b>			<b>1,884</b>	<b>606</b>
Senior unsecured convertible bonds <sup>1</sup>		2	60,000	0
<b>Noncurrent liabilities</b>			<b>60,000</b>	<b>0</b>
<b>Total liabilities</b>			<b>61,884</b>	<b>606</b>
Share capital		3.3	6,289	6,280
<i>Reserves from capital contributions</i>			7,450	7,425
<i>Other capital reserves</i>			2,916	2,916
Statutory capital reserves			10,366	10,341
<i>Accumulated result</i>			-13,752	-6,451
<i>Results carried forward</i>			-6,451	-5,557
<i>Net result for the period</i>			-7,301	-895
<i>Other voluntary reserves (free reserves)</i>			50,495	50,495
Voluntary accumulated result and other reserves			36,743	44,044
Treasury shares		3.4	-335	-172
<b>Total equity</b>			<b>53,063</b>	<b>60,493</b>
<b>Total liabilities and equity</b>			<b>114,947</b>	<b>61,099</b>

<sup>1</sup> interest bearing

## Income Statement

For the year ended December 31, in CHF thousands	<b>Notes</b>	<b>2017</b>	2016
Income from shareholdings	3.5	1,325	1,551
Other operating income		11	134
<b>Total operating income</b>		<b>1,336</b>	<b>1,685</b>
General and administrative expenses	3.6	-5,407	-2,159
Employee costs		-1,122	-792
Other operating expenses		-12	-25
<b>Total operating expenses</b>		<b>-6,541</b>	<b>-2,976</b>
<b>Operating result</b>		<b>-5,205</b>	<b>-1,291</b>
Financial income		1,011	358
Financial expenses		-3,189	-18
<b>Financial result</b>		<b>-2,178</b>	<b>340</b>
Reversal on allowance of investment		82	56
<b>Result before taxes</b>		<b>-7,301</b>	<b>-895</b>
Direct taxes		0	0
<b>Net result</b>		<b>-7,301</b>	<b>-895</b>

## Notes to the Statutory Financial Statements

### 1 Introduction

Santhera Pharmaceuticals Holding AG (the Company or Santhera) is the parent company of Santhera Group. The Company has its business offices at Hohenrainstrasse 24 in 4133 Pratteln, Switzerland. The legal domicile will remain at Hammerstrasse 49 in 4410 Liestal, Switzerland, until the forthcoming Annual General Meeting when the shareholders will vote on its relocation to Pratteln.

### 2 Principles

#### General

The statutory financial statements of the Company are prepared in accordance with the general accepted accounting principles as set out in Art. 957 to Art. 963b, of the Swiss Code of Obligations (**CO**). Since Santhera prepares consolidated financial statements in accordance with International Financial Reporting Standards (**IFRS**) of the International Accounting Standards Board (**IASB**), a recognized accounting standard, the Company has, in accordance with the CO, elected to forego presenting the statement of cash flows, the additional disclosures and the management report otherwise required by the CO.

#### Cash

Santhera holds cash balances, denominated mainly in Swiss francs (**CHF**) which include cash deposited in demand bank accounts, money market investment accounts and other liquid investments and interest earned on such cash balances.

#### Financial assets short-term

Financial assets (units in a fund) are held for trading and measured at fair value. In case of gains and losses from such assets are recognized through the income statement as financial income or financial expense.

#### Current assets and liabilities

Current assets are recorded at historical cost less adjustments for impairment of value and current liabilities at historical cost.

#### Loans to shareholdings

These are valued at their acquisition cost adjusted for impairment losses.

## Investments in shareholdings

Investments in shareholdings are recorded at acquisition cost less adjustments for impairment of value. Investments in subsidiaries are evaluated for impairment annually and an impairment loss is recorded when the carrying amount of such assets exceeds the fair value. Fair value estimates of investments are predominantly based on the income approach.

## Convertible bonds

On February 17, 2017, Santhera issued senior unsecured convertible bonds in the nominal amount of CHF 60 million. The bonds were issued at 100% of the principal amount and will also mature at 100% of that amount on February 17, 2022, unless previously redeemed, converted or repurchased and cancelled. The bonds, listed on the SIX, are interest bearing (5%) with a maximum term of 5 years and are convertible into registered Shares of Santhera with a nominal value of CHF 1 each. The initial conversion price is fixed at CHF 86.4006 and will be reset after the first year if the volume weighted average price (VWAP) of the Shares during a specified period of time will be below the reference share price (CHF 71.9969). The new conversion price must not be lower than 75% of the conversion price at issuance. In addition, Santhera may call the convertible bonds at any time on or after the second anniversary of the issue date at par, plus accrued interest, if any, if the VWAP of the Shares is at least 160% of the conversion price.

## Treasury shares

Treasury shares are recognized at acquisition cost and deducted from shareholders' equity at the time of acquisition. Santhera holds treasury shares for market making which is maintained by an external bank. In case of a resale, the gain or loss is recognized through the income statement as financial income or financial expenses.

## Related parties

In the meaning of the Swiss Accounting Law, related parties are only considered to be shareholders, direct and indirect subsidiaries (shareholdings) and the Board of Directors.

# 3 Information on Balance Sheet and Income Statement Items

## 3.1 Loans to shareholdings

Loans are granted to shareholdings primarily to fund the development and marketing activities of the Santhera Group (December 31, 2017: CHF 235.6 million; December 31, 2016: CHF 190.1 million). Until the end of 2015 the balance consisted of fully impaired and subordinated loans to Santhera Pharmaceuticals (Schweiz) AG. To finance the activities in development and the commercialization of LHON, in 2016 the loan granted to Santhera Pharmaceuticals (Schweiz) AG was increased (with the additional loans also being subordinated). As part of the annual reassessment as of December 31, 2017, Executive Management concluded that approximately 30% of the total loan balance is recoverable considering a more positive outlook, both in terms of market success of the developed and launched product (Raxone in LHON) and the development progress in other indications (e.g. idebenone in DMD).

### 3.2 Investments in shareholdings

In 2017 and 2016, the following companies are direct subsidiaries of Santhera Pharmaceuticals Holding AG (100% ownership and 100% voting rights):

	Share capital at December 31	2017	2016
Santhera Pharmaceuticals (Schweiz) AG Pratteln, Switzerland	CHF	125,000	125,000
Santhera Pharmaceuticals (Deutschland) GmbH Lörrach, Germany	EUR	25,000	25,000
Santhera Pharmaceuticals (USA), Inc. Burlington, US	USD	1,000	1,000
Santhera Pharmaceuticals (Canada), Inc. Montréal, Canada	CAD	1,000	1,000
Oy Santhera Pharmaceuticals (Finland) Ltd Helsinki, Finland	EUR	2,500	2,500

Santhera Pharmaceuticals (Schweiz) AG is the primary operational entity while Santhera Pharmaceuticals (Deutschland) GmbH holds the market authorization for the EU. Oy Santhera Pharmaceuticals (Finland) Ltd is not employing any personnel.

The following companies are 100% direct subsidiaries (100% voting rights) of Santhera Pharmaceuticals (Schweiz) AG:

	Share capital at December 31	2017	2016
Santhera Pharmaceuticals (Liechtenstein) AG Ruggell, Fürstentum Liechtenstein	CHF	50,000	50,000
Santhera (Italy) S.r.l. Milano, Italy	EUR	50,000	50,000
Santhera (Germany) GmbH München, Germany	EUR	50,000	50,000
Santhera (Netherlands) B.V. Nieuwegein, The Netherlands	EUR	50,000	50,000
Santhera (UK) Limited London, United Kingdom	GBP	50,000	50,000

### 3.3 Share capital

During 2017, the share capital was increased by a total amount of CHF 8,698 to CHF 6,288,555 as of December 31, 2017 (2016: CHF 6,279,857) through the exercise of employee stock options (from conditional share capital).

### 3.4 Treasury shares

The movement of treasury shares held by Santhera was as follows:

	No of Shares	TCHF
January 1, 2016	8,028	177
Purchase	23,002	1,069
Sale <sup>1</sup>	-27,414	-1,074
December 31, 2016	3,616	172
Purchase	180,083	9,567
Sale	173,778	-9,404
December 31, 2017	9,921	335

<sup>1</sup> In connection with the liquidation of Oy Juvantia Pharma, Turku, Finland (**Juvantia**), acquired in 2009, Santhera received 8,028 shares from former Juvantia shareholders. These treasury shares served as pledge from the former owners of Juvantia for compensation of a potential tax claim related to pre-acquisition activities. The claim was resolved and the shares were sold with a financial profit of TCHF 186.

### 3.5 Income from shareholdings

Income from shareholdings represents reimbursement for management services provided by the Company to its major shareholding Santhera Pharmaceuticals (Schweiz) AG.

### 3.6 General and administrative expenses

	In CHF thousands	2017	2016
Administrative expenses		1,220	990
Consulting expenses		1,456	1,169
Expenses in connection with convertible bonds		2,731	0
<b>Total</b>		<b>5,407</b>	<b>2,159</b>

## 4 Other Information

### 4.1 Full-time equivalents

The number of full-time equivalents at period end was not above 10 in 2017 and 2016.

### 4.2 Significant shareholders (>2%)

Pursuant to information from the Company's share register and the disclosure of participations made to the Company in accordance with applicable stock exchange regulation, the following shareholders owned 2% or more of the Company's share capital as registered in the commercial register at December 31, 2017: 6,279,857 shares (December 31, 2016: 6,262,798 shares):

	2017 Shares <sup>1</sup>	2017 %	2016 Shares <sup>2</sup>	2016 %
Iglu Group, Switzerland <sup>3</sup>	557,350	8.9	632,300	10.1
Bertarelli Ernesto, Guichard-Bertarelli Donata and Bertarelli Maria-Iris, Switzerland <sup>3</sup>	545,777	8.7	545,777	8.7
The Goldman Sachs Group, Inc., Corporation Trust Centre <sup>4</sup>	457,309	7.3	n/a	n/a
Roderick Wong (RTW Master Fund, LTD, US)	315,339	5.0	146,365	2.3
Norges Bank (the Central Bank of Norway) <sup>5</sup>	214,258	3.4	n/a	n/a
Consonance Capital Management, US	n/a	<3	597,069	9.5
UBS Fund Management (Switzerland) AG	n/a	<3	195,007	3.1
Lagoda Investments Management, LLC, US	n/a	<3	187,888	3.0
UBS Fund Management (Luxembourg) S.A.	n/a	<3	183,699	2.9
Union Asset Management Holding AG	n/a	<3	175,838	2.8
Visium Balanced Master Fund, Ltd., US <sup>6</sup>	n/a	n/a	n/a	n/a

<sup>1</sup> Including disclosures until December 31, 2017

<sup>2</sup> Including disclosures until December 31, 2016

<sup>3</sup> On January 25, 2017, both Iglu Group and the Bertarelli Group announced that they had formed a new group with combined holdings of 1,179,977 Shares (18.8%).

<sup>4</sup> Purchase positions in connection with securities lending transactions (as of January 3, 2018)

<sup>5</sup> Purchase positions in connection with securities lending transactions, shares held as collateral (as of January 17, 2018)

<sup>6</sup> The fund was liquidated in June 2016 (Bloomberg, June 18, 2016).

### 4.3 Disclosure of shares and equity rights (share appreciation rights and stock options) held by members of the Board and Executive Management (and their respective related party)

As of December 31, 2017:

	Number of Shares	Number of vested equity rights	Number of unvested equity rights	Total number of equity rights
<i>Board of Directors</i>				
Elmar Schnee, Chairman since April 4, 2017	2,000	0	4,486	4,486
Martin Gertsch, Vice-Chairman	38,109	1,500	8,935	10,435
Jürg Ambühl, Director until April 4, 2017 <sup>1</sup>	30,000	7,281	0	7,281
Philipp Gutzwiller, Director since April 2017	500	0	3,157	3,157
Thomas Meier, Director since April 4, 2017		--- See below ---		
Patrick Vink, Director since April 4, 2017	1,000	0	6,116	6,116
<i>Executive Management</i>				
Thomas Meier, CEO	75,562	8,000	29,663	37,663
Todd Bazemore, Chief Operating Officer US until November 17, 2017 <sup>2</sup>	0	0	0	0
Nicholas Coppard, Head Development until January 31, 2017 <sup>3</sup>	0	3,500	19,216	22,716
Günther Metz, Head Business Development	0	14,000	16,500	30,500
Christoph Rentsch, Chief Financial Officer	0	7,500	28,601	36,101
Kristina Sjöblom Nygren, Chief Medical Officer & Head Development since January 1, 2017	0	0	18,617	18,617
Giovanni Stropoli, Chief Commercial Officer Europe and Rest of World	250	7,500	26,499	33,999
Oliver Strub, General Counsel and Secretary to the Board	0	6,001	16,990	22,991

<sup>1</sup> Number of Shares as of April 4, 2017

<sup>2</sup> Number of Shares as of November 17, 2017

<sup>3</sup> Number of Shares as of January 31, 2017

As of December 31, 2016:

	Number of Shares	Number of vested equity rights	Number of unvested eq- uity rights	Total number of equity rights
<i>Board of Directors</i>				
Martin Gertsch, Chairman	38,109	0	6,281	6,281
Jürg Ambühl, Director	30,000	0	7,281	7,281
<i>Executive Management</i>				
Thomas Meier, CEO	72,902	3,750	14,875	18,625
Todd Bazemore, Chief Operating Officer US <sup>1</sup>	0	0	34,881	34,881
Nicholas Coppard, Head Development	0	0	12,250	12,250
Günther Metz, Head Business Development	0	12,000	7,120	19,120
Christoph Rentsch, Chief Financial Officer	0	0	22,000	22,000
Giovanni Stropoli, Chief Commercial Officer Europe and Rest of World	600	0	20,565	20,565
Oliver Strub, General Counsel and Secretary to the Board	0	9,001	7,240	16,241

<sup>1</sup> Joined the Executive Management on September 6, 2016.

#### 4.4. Disclosure of the allocation of equity rights for Board of Directors, Executive Management and employees of Santhera Group

	2017 Quantity	2017 Value (in TCHF) <sup>1</sup>	2016 Quantity	2016 Value (in TCHF) <sup>1</sup>
Board of Directors	17,913	524	6,562	224
Executive Management	104,033	3,686	65,431	1,349
Employees of Santhera Group	228,297	4,876	126,980	3,121
<b>Total</b>	<b>350,243</b>	<b>9,086</b>	<b>198,973</b>	<b>4,694</b>

<sup>1</sup> Value of the equity rights calculated in accordance with the Hull-White model at the date of allocation in accordance with the terms of the award. The tax value of equity rights is 0 until they would be exercised. Such equity rights values are theoretical values and do not reflect income tax values and do also take into consideration certain vesting provisions. For information about the underlying equity rights plans, see note 17 "Equity Rights Plans" in the consolidated financial statements. For information about the Company's compensation procedures, consult the Corporate Governance Report and the Compensation Report.

On January 1, 2018, 444,164 share appreciation rights (**SARs**) were planned to be granted to employees of Santhera (conditionally for the Executive Management). These SARs are part of the bonus award for the year 2017 to employees of the Group. These SARs were granted under ESARP 2016 (see note 17 "*Equity Rights Plans*").

	Quantity	Value (in TCHF) <sup>1</sup>
Executive Management	103,810	1,452
Employees of Santhera Group	340,354	4,761
<b>Total</b>	<b>444,164</b>	<b>6,213</b>

<sup>1</sup> Value of the equity rights calculated in accordance with the Hull-White model at the date of allocation in accordance with the terms of the award. The tax value of equity rights is 0 until they would be exercised. Such equity rights values are theoretical values and do not reflect income tax values and do also take into consideration certain vesting provisions. For information about the underlying equity rights plans, see note 17 "*Equity Rights Plans*" in the consolidated financial statements. For information about the Company's compensation procedures, consult the Corporate Governance Report and the Compensation Report.

#### 4.5 Contingencies and guarantees

##### *Guarantee towards Swiss VAT authorities*

The Company is part of the value-added tax group of the Swiss affiliated companies of Santhera Pharmaceuticals and is therefore jointly and severally liable to the Swiss federal tax administration for their value-added tax liabilities.

##### *Guarantee towards Santhera Pharmaceuticals (Schweiz) AG*

The Company guarantees to pay for the liabilities of its subsidiary Santhera Pharmaceuticals (Schweiz) AG until the Annual General Meeting in 2018.

##### *Declaration of liability towards Arval Deutschland GmbH*

The Company guarantees to pay for the liabilities of its subsidiary Santhera (Germany) GmbH for contractual duties and obligations.

#### 4.6 Events after the reporting date

On January 26, 2017, Santhera announced that the Committee for Medicinal Products for Human Use (**CHMP**) of the European Medicines Agency (**EMA**) maintained its negative opinion on the Type II extension application for idebenone in Duchenne muscular dystrophy (**DMD**) following a re-examination procedure. The CHMP concluded that an approval for idebenone in DMD, applied as a Type II variation of the existing marketing authorization, cannot be granted at the present time based on the current existing evidence. Although the positive outcome of the Phase III DELOS trial was acknowledged, the CHMP has invited Santhera to present additional data to further link the observed treatment effects on respiratory function outcomes to patient benefit. Management has considered the impact on the Group's ability to continue as a going concern and potential indicators of impairment on the financial statements as of December 31, 2017.

On February 15, 2018, Santhera announced that it has entered into a license agreement with Polyphor Ltd., Allschwil, Switzerland, for POL6014, a clinical stage selective inhibitor of human neutrophil elastase with the potential to treat cystic fibrosis (**CF**) and other neutrophilic pulmonary diseases such as non-cystic fibrosis bronchiectasis (**NCFB**), alpha-1 antitrypsin deficiency (**AATD**) and primary ciliary dyskinesia (**PCD**).

Santhera will assume the global development, regulatory filings and commercialization of POL6014. The development program has been advanced with financial support by the Cystic Fibrosis Foundation Therapeutics Inc. (**CFFT**), USA, to Polyphor. With POL6014, Santhera is expanding its product pipeline in pulmonary diseases where the Company is already developing its lead product idebenone for respiratory complication in Duchenne muscular dystrophy.

Under the agreement, Santhera obtains the worldwide, exclusive rights to develop and commercialize POL6014, an innovative macrocycle elastase inhibitor, and analogs for an initial payment of CHF 6.5 million, payable in Santhera shares at an agreed valuation of CHF 27.2053 per share and additional cash payments of up to CHF 121 million contingent to future development, regulatory and particularly sales milestones. In addition, Polyphor is entitled to tiered royalty payments from Santhera's future net sales of POL6014 and to undisclosed milestone payments and royalties provided that Santhera advances the development and market entry of POL6014 in other pulmonary diseases. Santhera issued the 238,924 shares (3.8% of its currently issued shares) required for the initial payment to Polyphor out of its existing authorized share capital.



Ernst & Young Ltd  
Aeschengraben 9  
P.O. Box  
CH-4002 Basle

Phone +41 58 286 86 86  
Fax +41 58 286 86 00  
www.ey.com/ch

To the General Meeting of  
Santhera Pharmaceuticals Holding Ltd, Liestal

Basle, March 19, 2018

## Report of the statutory auditor on the financial statements

As statutory auditor, we have audited the financial statements of Santhera Pharmaceuticals Holding Ltd, which comprise the balance sheet, income statement and notes (pages 63 to 73), for the year ended December 31, 2017.



### Board of Directors' responsibility

The Board of Directors is responsible for the preparation of the financial statements in accordance with the requirements of Swiss law and the company's articles of incorporation. This responsibility includes designing, implementing and maintaining an internal control system relevant to the preparation of financial statements that are free from material misstatement, whether due to fraud or error. The Board of Directors is further responsible for selecting and applying appropriate accounting policies and making accounting estimates that are reasonable in the circumstances.



### Auditor's responsibility

Our responsibility is to express an opinion on these financial statements based on our audit. We conducted our audit in accordance with Swiss law and Swiss Auditing Standards. Those standards require that we plan and perform the audit to obtain reasonable assurance whether the financial statements are free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the financial statements. The procedures selected depend on the auditor's judgment, including the assessment of the risks of material misstatement of the financial statements, whether due to fraud or error. In making those risk assessments, the auditor considers the internal control system relevant to the entity's preparation of the financial statements in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the entity's internal control system. An audit also includes evaluating the appropriateness of the accounting policies used and the reasonableness of accounting estimates made, as well as evaluating the overall presentation of the financial statements. We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our audit opinion.



### Opinion

In our opinion, the financial statements for the year ended December 31, 2017 comply with Swiss law and the company's articles of incorporation.



### Report on key audit matters based on the circular 1/2015 of the Federal Audit Oversight Authority

Key audit matters are those matters that, in our professional judgment, were of most significance in our audit of the financial statements of the current period. These matters were addressed in the context of our audit of the financial statements as a whole, and in forming our opinion thereon, and we do not provide a separate opinion on these matters. For each matter below, our description of how our audit addressed the matter is provided in that context.

We have fulfilled the responsibilities described in the *Auditor's responsibilities* section of our report, including in relation to these matters. Accordingly, our audit included the performance of procedures designed to respond to our assessment of the risks of material misstatement of the financial statements. The results of our audit procedures, including the procedures performed to address the matters below, provide the basis for our audit opinion on the financial statements.

#### Management's assessment of the Group's ability to continue as a going concern

---

**Area of focus** Based on the stage of the operations combined with recurring development costs and the level of cash inflows from its operating activities, the Group incurred negative cash flows from operations in the past and it expects to continue to incur such costs and negative cash flows in the near future. Management has prepared various budget scenarios and business plans and has assessed the likelihood and impact that uncertain cash flows could have on the Group's ability to continue as going concern. This assessment was based on the evaluation of the available year-end funds of CHF 45.2 million as well as the budget.

Despite the Group's development into a more sales oriented business, funding from non-revenue related sources is still required in the future and we therefore concluded that the assessment of the ability to continue as a going concern represents a key audit matter for our audit.

---

**Our audit response** Our audit procedures related to the key audit matter of the assessment of the going concern included the following procedures:

- We gained an understanding of management's budgeting and forecasting process underlying the going concern assessment.
- We discussed the budget and business plans with management, evaluated the development and the assessments made and also considered how uncertain elements were included in the budget and business plans. We evaluated underlying key assumptions such as expected cash inflow from product sales and expected cash outflow from purchases of inventory, research and development expenses and other operating expenses.
- We tested the sensitivity of the revenue estimates and assessed how they compared to historical revenues.
- We further discussed the base case scenario with management and assessed the likelihood of this scenario.

- We performed inquiries of management about current developments and the ability to execute the elements included in the budget and business plans supporting the evaluation of the going concern assumption. We read the Board of Directors meeting minutes that approved the underlying budget to assess potential contrary information. We performed procedures regarding subsequent events to assess the accuracy of the budget as of the date of the auditor's report.

### Valuation of investments in and long-term receivables from shareholdings

**Area of focus** Santhera Pharmaceuticals Holding Ltd holds investments in subsidiaries and grants loans to subsidiaries for financing purposes, both of which are assessed for impairment as of the balance sheet date. Management's assessment requires estimation and judgement around assumptions used, including prospective financial information and discount rates. Changes to assumptions could lead to significant changes in the estimated recoverable amount, impacting both potential impairment charges as well as potential reversals of impairment. As such, we considered this matter to be significant to our audit.

*Refer to note 3.1 and 3.2 to these financial statements for Santhera Pharmaceuticals Holding Ltd disclosures related to investment in and long-term receivables from shareholdings.*

**Our audit response** We evaluated management's impairment assessment, which is based on an income approach, and analysed the underlying key assumptions in relation to prospective financial information as well as discount rates used. We evaluated the historical accuracy of the Company's previous estimates on prospective financial information. We tested the sensitivity of the assessment due to changes to key assumptions and compared these assumptions to externally available information in order to assess management's impairment conclusion.



### Report on other legal requirements

We confirm that we meet the legal requirements on licensing according to the Auditor Oversight Act (AOA) and independence (article 728 CO and article 11 AOA) and that there are no circumstances incompatible with our independence.

In accordance with article 728a para. 1 item 3 CO and Swiss Auditing Standard 890, we confirm that an internal control system exists, which has been designed for the preparation of financial statements according to the instructions of the Board of Directors.

We recommend that the financial statements submitted to you be approved.

Ernst & Young Ltd

/s/ Frederik Schmachtenberg  
Licensed audit expert  
(Auditor in charge)

/s/ Jan Meyer  
Licensed audit expert

# Consolidated Financial Statements

## Contents

Consolidated Balance Sheet .....	18
Consolidated Income Statement .....	19
Consolidated Statement of Comprehensive Income .....	20
Consolidated Cash Flow Statement .....	21
Consolidated Statement of Changes in Equity .....	22
Notes to the Consolidated Financial Statements .....	23
1 General Information .....	23
2 Summary of Significant Accounting Policies .....	23
3 Critical Accounting Estimates, Assumptions and Judgments .....	31
4 Exchange Rates of Principal Currencies .....	31
5 Tangible Assets .....	32
6 Intangible Assets .....	33
7 Impairment Test for Intangible Assets .....	34
8 Prepaid Expenses and Other Assets .....	34
9 Inventories .....	35
10 Trade and Other Receivables .....	35
11 Cash and Cash Equivalents .....	35
12 Share Capital .....	36
13 Deferred Taxes .....	38
14 Trade and Other Payables .....	39
15 Accrued Expenses .....	39
16 Commitments and Contingent Liabilities .....	39
17 Equity Rights Plans .....	41
18 Segment and Geographic Information .....	47
19 Other Operating Income .....	48
20 Operating Expenses by Nature .....	48

21	Employee Expenses and Benefits .....	48
22	Financial Income/Expenses .....	52
23	Income Taxes .....	52
24	Earnings/Loss per Share .....	53
25	Related Party Transactions .....	53
26	Risk Management Objectives and Policies .....	54
27	Events After the Reporting Date .....	57
	Statutory Auditor's Report on the Audit of the Consolidated Financial Statements .....	59

## Consolidated Balance Sheet

	In CHF thousands	Notes	31.12.2016	31.12.2015
<b>Assets</b>				
Tangible assets		5	517	398
Intangible assets		6	26,549	29,559
Financial assets long-term			270	190
Deferred tax assets		13	1,106	3,061
<b>Noncurrent assets</b>			<b>28,442</b>	<b>33,208</b>
Prepaid expenses and other assets		8	583	1,513
Inventories		9	7,676	3,441
Trade and other receivables		10	4,276	2,131
Cash and cash equivalents		11	49,815	76,859
<b>Current assets</b>			<b>62,350</b>	<b>83,944</b>
<b>Total assets</b>			<b>90,792</b>	<b>117,152</b>
<b>Equity and liabilities</b>				
Share capital		12	6,280	6,263
Capital reserves and share premium			382,322	377,031
Retained earnings			-308,549	-273,133
Employee benefit reserve			-4,734	-2,958
Treasury shares		12	-172	-177
Other components of equity			-796	-779
<b>Total equity</b>			<b>74,351</b>	<b>106,247</b>
Pension liabilities		21	6,183	3,957
<b>Total noncurrent liabilities</b>			<b>6,183</b>	<b>3,957</b>
Trade and other payables		14	4,458	3,666
Accrued expenses		15	5,800	3,282
<b>Total current liabilities</b>			<b>10,258</b>	<b>6,948</b>
<b>Total liabilities</b>			<b>16,441</b>	<b>10,905</b>
<b>Total equity and liabilities</b>			<b>90,792</b>	<b>117,152</b>

## Consolidated Income Statement

For the year ended December 31, in CHF thousands	Notes	2016	2015
Net sales	18	19,033	4,321
Cost of goods sold		-3,883	-1,371
<i>Of which amortization intangible asset</i>		-3,039	-1,013
Other operating income	19	361	188
Development	20	-17,675	16,651
<i>Of which Development expenses</i>	20	-17,675	-10,453
<i>Of which reversal impairment on intangible assets and inventory</i>	20	0	27,104
Marketing and sales	20	-21,051	-8,356
General and administrative	20	-9,805	-8,244
Other operating expenses	20	-107	-16
<b>Operating expenses</b>	<b>20</b>	<b>-48,638</b>	<b>35</b>
<b>Operating result</b>		<b>-33,127</b>	<b>3,173</b>
Financial income	22	928	416
Financial expenses	22	-995	-655
<b>Result before taxes</b>		<b>-33,194</b>	<b>2,934</b>
Income taxes	23	-2,221	3,015
<b>Net result</b>		<b>-35,415</b>	<b>5,949</b>
Basic earnings/loss per share (in CHF)	24	-5.65	1.11
Diluted earnings/loss per share (in CHF)	24	-5.65	1.08

## Consolidated Statement of Comprehensive Income

For the year ended December 31, in CHF thousands	Notes	2016	2015
<b>Net result</b>		<b>-35,415</b>	<b>5,949</b>
<i>Items never to be reclassified to net income in subsequent periods:</i>			
Actuarial gains/(losses) on defined benefit plans	21	-1,776	-1,671
<i>Items to be reclassified to net income in subsequent periods:</i>			
Currency translation differences		-18	-16
<b>Other comprehensive result</b>		<b>-1,794</b>	<b>-1,687</b>
<b>Total comprehensive result</b>		<b>-37,209</b>	<b>4,262</b>

## Consolidated Cash Flow Statement

For the year ended December 31, in CHF thousands	Notes	2016	2015
Result before taxes		<b>-33,194</b>	<b>2,934</b>
Depreciation of tangible assets	5	168	85
Reversal of impairment on intangible assets	6	0	-26,157
Amortization of intangible assets	6	3,096	1,037
Expenses for equity rights plans	17, 20	4,683	2,040
Change in pension liabilities	21	450	-394
Taxes paid		-266	-46
Change in net working capital		-2,131	-2,119
Total financial result	22	67	239
Interest received	22	5	2
Interest paid	22	-15	-11
<b>Cash flow from operating activities</b>		<b>-27,137</b>	<b>-22,390</b>
Investments in tangible assets	5	-289	-350
Investments in intangible assets	6	-86	-165
Investments in other financial assets		-84	-104
<b>Cash flow from investing activities</b>		<b>-459</b>	<b>-619</b>
Capital increases from options exercised	12	385	2,127
Proceeds from sale of treasury shares	12	418	0
Purchase of treasury shares	12	-172	0
Capital increase private placement	12	0	54,870
Capital increase	12	0	27,576
Cost of issuance of share capital		0	-1,943
<b>Cash flow from financing activities</b>		<b>631</b>	<b>82,630</b>
Effects of exchange rate changes on cash and cash equivalents		-79	-197
<b>Net increase/(decrease) in cash and cash equivalents</b>		<b>-27,044</b>	<b>59,424</b>
Cash and cash equivalents at January 1		76,859	17,435
<b>Cash and cash equivalents at December 31</b>		<b>49,815</b>	<b>76,859</b>

## Consolidated Statement of Changes in Equity

In CHF thousands	Notes	Share capital	Capital reserves and share premium	Retained earnings	Employee benefit reserve	Treasury shares	Translation differences	Total
<b>Balance at January 1, 2015</b>		<b>4,974</b>	<b>293,650</b>	<b>-279,083</b>	<b>-1,287</b>	<b>-177</b>	<b>-762</b>	<b>17,315</b>
Net result		0	0	5,949	0	0	0	5,949
Other comprehensive result	21	0	0	0	-1,671	0	-16	-1,687
<b>Total comprehensive result for the period</b>		<b>0</b>	<b>0</b>	<b>5,949</b>	<b>-1,671</b>	<b>0</b>	<b>-16</b>	<b>4,262</b>
Share-based payment transactions	17, 20	0	2,040	0	0	0	0	2,040
Capital increase from options exercise	12	399	1,728	0	0	0	0	2,127
Capital increase private placement	12	590	54,280	0	0	0	0	54,870
Capital increase	12	300	27,276	0	0	0	0	27,576
Cost of issuance of share capital		0	-1,943	0	0	0	0	-1,943
<b>Balance at December 31, 2015</b>		<b>6,263</b>	<b>377,031</b>	<b>-273,134</b>	<b>-2,958</b>	<b>-177</b>	<b>-778</b>	<b>106,247</b>
<b>Balance at January 1, 2016</b>		<b>6,263</b>	<b>377,031</b>	<b>-273,134</b>	<b>-2,958</b>	<b>-177</b>	<b>-778</b>	<b>106,247</b>
Net result		0	0	-35,415	0	0	0	-35,415
Other comprehensive result	21	0	0	0	-1,776	0	-18	-1,794
<b>Total comprehensive result for the period</b>		<b>0</b>	<b>0</b>	<b>-35,415</b>	<b>-1,776</b>	<b>0</b>	<b>-18</b>	<b>-37,209</b>
Share-based payment transactions	17, 20	0	4,682	0	0	0	0	4,682
Capital increase from options exercise	12	17	368	0	0	0	0	385
Change in treasury shares	12	0	241	0	0	5	0	246
<b>Balance at December 31, 2016</b>		<b>6,280</b>	<b>382,322</b>	<b>-308,549</b>	<b>-4,734</b>	<b>-172</b>	<b>-796</b>	<b>74,351</b>

# Notes to the Consolidated Financial Statements

## 1 General Information

Santhera Pharmaceuticals Holding AG (the **Company**, together with its subsidiaries **Santhera** or **Group**) is a specialty pharmaceutical company focused on the development and commercialization of products for the treatment of mitochondrial and neuromuscular diseases, an area which includes many orphan and niche indications with high unmet medical need.

The Company, having its primary listing of its registered shares (**Shares**) on the SIX Swiss Exchange (**SIX**), is a Swiss stock corporation and the parent company of the Group. Its purpose is to acquire, dispose and manage investments. The Company has its registered offices at Hammerstrasse 49 in 4410 Liestal, Switzerland.

The consolidated financial statements were approved for publication by the Board of Directors (**Board**) on March 6, 2017. They are subject to approval by the Annual Shareholders' Meeting (**ASM**) on April 4, 2017.

## 2 Summary of Significant Accounting Policies

The principal accounting policies applied in the preparation of these financial statements are set out below. These policies have been consistently applied to all the years presented, unless otherwise stated.

### Basis of preparation

The consolidated financial statements of Santhera have been prepared in accordance with International Financial Reporting Standards (**IFRS**).

The consolidated financial statements are based on the financial statements of the individual Santhera companies prepared for the same reporting period using consistent accounting policies. The consolidated financial statements are prepared using the historical cost convention except for the revaluation to fair value of certain financial assets and financial liabilities.

The presentation currency is Swiss francs (**CHF**). All figures included in these financial statements and notes to the financial statements are rounded to the nearest CHF 1,000 except where otherwise indicated.

## Consolidation

Subsidiaries in which the Company has a direct or indirect controlling interest are consolidated. Control exists when the investor is exposed, or has rights, to variable returns from its investment with the investee and has the ability to affect those returns through its power over the investee. Control is normally evidenced when the Company owns, either directly or indirectly, more than 50% of the voting rights or potential voting rights of a company's share capital that are currently exercisable.

The consolidated financial statements of Santhera include the accounts of Santhera Pharmaceuticals Holding AG, Liestal, Switzerland, and its wholly owned subsidiaries Santhera Pharmaceuticals (Schweiz) AG, Liestal, Switzerland; Santhera Pharmaceuticals (USA), Inc., Burlington, US; Santhera Pharmaceuticals (Canada), Inc., Montréal, Canada; Santhera Pharmaceuticals (Deutschland) GmbH, Lörrach, Germany; and Oy Santhera Pharmaceuticals (Finland) Ltd, Helsinki, Finland. The accounts further include the wholly owned subsidiaries of Santhera Pharmaceuticals (Schweiz) AG: Santhera Pharmaceuticals (Liechtenstein) AG, Ruggell, Fürstentum Liechtenstein; Santhera (Italy) S.r.l., Milano, Italy; Santhera (Germany) GmbH, Munich, Germany; Santhera (Netherlands) B.V., Nieuwegein, The Netherlands; and Santhera (UK) Limited, London, United Kingdom.

Consolidation commences from the date on which control is transferred to the Company, and subsidiaries are no longer consolidated from the date that control ceases. Intercompany balances and transactions between Group companies are eliminated. Intercompany transactions solely result from providing services, financing and selling goods to other Group companies.

## Changes in accounting policies

The adopted accounting policies are consistent with the previous year except for those described below.

### *New, revised or amended IFRS standards and interpretations 2016*

The following new, revised or amended standards that became effective on January 1, 2016 did not have any significant impact on the consolidated financial statements.

- IAS 1 (Amendments) Disclosure Initiative
- IAS 16 and IAS 38 (Amendments) Clarification of Acceptable Methods of Depreciation and Amortization
- Annual Improvements to IFRSs 2012–2014 Cycle

### *New, revised or amended IFRS standards and interpretations as from 2017*

The following new, revised or amended standards have been published but are not yet effective and have not been early adopted by the Group.

- IFRS 9 Financial Instruments (effective January 1, 2018)
- IAS 7 (Amendments) Disclosure Initiative (effective January 1, 2017)
- IAS 12 (Amendments) Recognition of Deferred Tax Assets for Unrealized Losses (effective January 1, 2017)
- IFRS 2 (Amendments) Classification and Measurement of Share-based Payment Transactions (effective January 1, 2018)

At this stage, the Group does not expect any significant impact on the consolidated financial statements from the new, revised or amended standards above, with the exception for the following standards set out below:

- IFRS 15 Revenue from Contracts with Customers (effective January 1, 2018). The Group has identified one revenue stream from its contracts with customers: product revenue. The evaluation of the contracts is ongoing with the primary focus on its consideration of the standard in regards to estimating the transaction price. While the Company has not completed the analysis of the impact of adoption, the adoption of IFRS 15 is not expected to have material effects on the consolidated financial statements. As part of the Company's analysis, the Company is evaluating and implementing changes to its policies, procedures and controls.
- IFRS 16 Leases (effective January 1, 2019). The lessee shall recognize leasing obligations in its balance sheet for future lease payments as well as recognizing a right to use the underlying asset. Santhera expects that the new standard IFRS 16 has an impact on the consolidated financial statements. However, a thorough estimate of the impact can only be made once a detailed analysis is finished.

### **Segment reporting**

Santhera has one operating segment, namely the development and commercialization of products for the treatment of mitochondrial and neuromuscular diseases. The Board, the Executive Management and senior managers, being the Chief Operating Decision Makers (**CODM**), assess the reporting data and allocate resources as one segment on an aggregated consolidated level according to operating expenses by function. Santhera generates revenue from sales of Raxone® for the treatment of LHON. Geographic revenue information is based on location of the customer or licensee.

### **Foreign currency translations**

The consolidated financial statements are presented in CHF. The functional currency of each of Santhera's companies is the currency of the primary economic environment in which the local entity operates. Transactions in foreign currencies are accounted for at the rates prevailing at the dates of the transaction. Translation differences from financial transactions are included in the financial result.

Gains and losses resulting from the translation of foreign currency transactions and from the adjustment of foreign currency monetary assets and liabilities at the reporting date are recognized in the income statement.

Assets and liabilities of foreign entities are translated into CHF using the balance-sheet exchange rates at year-end. Income and expenses are translated into CHF at average exchange rates. The exchange differences arising on the retranslation are accounted for in the statements of comprehensive income/equity.

## Intangible assets

Patents, licenses, trademarks and other intangible assets are capitalized as intangible assets when it is probable that future economic benefits will be generated. Such assets are in general amortized on a straight-line basis over their useful lives. Estimated useful life is the lower of legal duration or economic useful life. The estimated useful life of the intangible assets is regularly reviewed and if necessary, the future amortization charge is accelerated. For pharmaceutical products, the estimated useful life normally corresponds to the remaining lifetime of their patent or orphan drug protection (up to 20 years).

## Patents

Patents not yet available for use are not amortized, but tested for impairment annually. Once useful life can be determined, amortization starts on a straight-line basis (up to 20 years).

## IT software

Acquired IT software licenses are capitalized on the basis of the costs incurred to acquire and implement the specific software. These costs are amortized on a straight-line basis over their estimated useful lives (2 to 5 years).

## Tangible assets

Tangible assets are stated at cost less accumulated depreciation and any impairment losses. Depreciation is calculated on a straight-line basis over the estimated useful life of the asset or the shorter lease term, as follows:

	Useful life
Equipment	4 to 10 years
IT hardware	2 to 5 years
Leasehold improvements	2 to 10 years

## Impairment of assets

Assets include intangible assets not yet available for use, intangible assets with finite useful lives and tangible assets. In general and in accordance with the terms of IFRS, assets not in use are capitalized at cost in the balance sheet and reviewed for impairment at least annually. Impairment testing is performed at the same time every year or whenever there is an indication that the asset may be impaired. A change to finite useful life is accounted for as a change in an accounting estimate for the respective asset. Testing for indicators of impairment is done at the end of each reporting period.

## Trade and other receivables

Receivables which generally have 30 to 60 days payment terms are stated at their nominal value less an allowance for any uncollectible amount if required. An allowance for doubtful debts is made when collection is deemed no longer probable.

## **Inventories**

Inventories are stated at the lower of cost or net realizable value using the weighted average cost formula.

## **Financial assets**

Generally, Santhera classifies its financial assets in the following categories:

### *Financial assets at fair value through profit or loss*

This category has two subcategories: financial assets held for trading and those designated at fair value through profit or loss upon initial recognition. A financial asset is classified in this category if acquired principally for the purpose of selling in the short term. Assets in this category are classified as current assets if they are either held for trading or are expected to be realized within 12 months of the reporting date. Valuation is at fair value through profit and loss. Financial assets at fair value through profit or loss are subsequently carried at fair value. Realized and unrealized gains and losses arising from changes in the fair value are included in the income statement in the period in which they arise.

### *Loans and receivables*

Loans and receivables are nonderivative financial assets with fixed or determinable payments that are not quoted in an active market. They arise when Santhera provides money, goods or services directly to a debtor with no intention of trading the receivable. They are included in current assets, except for maturities longer than 12 months after the balance sheet date. These are classified as non-current assets. Loans and receivables are measured at amortized cost using the effective interest method.

## **Leases**

Leases of assets under which Santhera essentially assumes all the rewards and risks of ownership are classified as finance leases. Finance leases are capitalized as assets and liabilities at the commencement of the lease at the fair value of the leased item or, if lower, at the present value of the minimum lease payments. The assets acquired under these contracts are depreciated over the shorter of the estimated useful life of the asset or the lease term.

Leases of assets under which the risks and rewards of ownership are effectively retained by the lessor are classified as operating leases, and payments made are charged to the income statement on a straight-line basis.

## **Cash and cash equivalents**

This item includes cash on hand and at banks, deposits held at call with banks and other short-term highly liquid investments with original maturities of three months or less.

## **Share capital**

Common shares are classified as equity. Incremental costs directly attributable to the issue of new common shares or options are shown in equity in the capital reserves and share premium as a deduction, net of tax, from the proceeds.

## **Treasury shares**

Treasury shares are purchased at cost and recognized as deduction from equity. Income or loss from subsequent sale is presented in equity.

## **Financial liabilities**

Santhera classifies its financial liabilities into two categories:

### *Financial liabilities at fair value through profit or loss*

This category includes derivatives with negative replacement values. They are initially recognized at their fair value. Any subsequent change in fair value is recognized in the income statement in the period the changes occur.

### *Other liabilities measured at amortized costs*

This category principally covers debt instruments and trade and other payables. They are initially recognized at fair value and subsequently measured at amortized costs using the effective interest method. Any difference between the net proceeds received and the principal value due on redemption is amortized over the duration of the debt instrument and is recognized as part of interest expense in the income statement.

## **Income taxes**

The income tax charge is based on profit for the year and includes deferred taxes. Deferred taxes are calculated using the liability method. Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Deferred tax assets and liabilities are measured using the tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled based on enacted or substantially enacted tax rates as of the balance sheet date.

The amount of deferred tax liabilities and deferred tax assets reflects the tax consequences on the balance sheet date of the Company's expectation of recovery or settlement of such carrying amount of its assets and liabilities.

Deferred tax assets and liabilities are not discounted and are classified as noncurrent assets (liabilities) in the balance sheet. They are offset against each other if they relate to the same taxable entity and tax authority.

Deferred tax assets are recognized when it is probable that sufficient taxable profits will be available against which the deferred tax assets can be utilized. At each balance sheet date, the Company reassesses unrecognized deferred tax assets and the carrying amount of deferred tax assets. The Company recognizes a previously unrecognized deferred tax asset to the extent that it has become probable that future taxable profit will allow the deferred tax asset to be recovered. The Company conversely reduces the carrying amount of a deferred tax asset to the extent that it is no longer probable that sufficient taxable profit will be available to allow the benefit of part or the entire deferred tax asset to be utilized. Deferred tax is provided on temporary differences arising on investments in subsidiaries, associates and joint ventures, except where the timing of the reversal of the temporary difference can be controlled and it is probable that the difference will not reverse in the foreseeable future.

## Earnings/loss per share

Basic earnings/loss per share are calculated by dividing the net profit/loss attributable to owners of ordinary Shares of the Company by the weighted average number of Shares outstanding during the reporting period. Diluted earnings per share are calculated by dividing the net profit attributable to owners of ordinary Shares of the Company by the weighted average number of shares issued and outstanding during the reporting period adjusted for Shares held as treasury shares (purchased at market) and the number of potential shares from stock option plans.

## Employee benefits

### *Post-retirement benefits*

Santhera operates both defined benefit and defined contribution pension schemes.

- **Defined benefit scheme:**

Santhera's pension plan in Switzerland is classified as a defined benefit plan. Payments under this scheme are made directly to the pension fund for the account of each insured person. Typically, on retirement, an employee will receive an amount of the accumulated defined benefit obligation depending on several factors such as the total individual amount paid in, age and implied life expectancy. The compensation will be in the form of a lifelong pension or a lump sum payment. The scheme also covers disability as a consequence of illness and death-in-service.

The liability recognized in the balance sheet in respect of defined benefit pension plans is the present value of the defined benefit obligation at the balance sheet date less the fair value of plan assets, adjusted for the effects of the asset ceiling, when relevant.

The defined benefit obligation is calculated annually by independent actuaries using the projected unit credit method. The present value of the defined benefit obligation is determined by discounting the estimated future cash outflows using interest rates of high-quality corporate bonds that are denominated in the currency in which the benefits will be paid and that have terms to maturity approximating the terms of the related pension liability.

- **Defined contribution schemes:**

Defined contribution schemes are also funded through direct payments for the account of each insured person. Upon retirement, an employee will receive an amount of the accumulated contributions in the form of a lifelong pension or a lump sum payment. No further obligations arise from these schemes other than the fixed periodic contributions to the plan.

### *Share-based compensation*

Santhera has established stock option and share appreciation rights (**SARs**) plans to align the long-term interests of the members of the Board, the Executive Management, employees and selected consultants who are eligible to participate. Under all plans, options and share appreciation rights are equity-settled. The fair value of options and SARs is determined at the grant date and recognized as personnel expense over the period Santhera receives services for each award. Where stock option awards are modified as a minimum, the expenses are recognized as if no terms had been modified; modifications which increase the fair value of options are expensed additionally. Unless determined otherwise by the Board, terminations of employment by the employer are treated as forfeiture and any previously accumulated share-based payment expenses for unvested awards are reversed.

## **Provisions**

Provisions are recognized when Santhera has a present obligation (legal or constructive) as a result of a past event, where it is more probable than not that a cash outflow will be required to fulfill the obligation and where a reliable estimate can be made of the amount of the obligation.

If the effect of the time value of money is material, provisions are determined by discounting the expected future outflows.

## **Revenue recognition**

Revenue comprises the fair value of the sale of goods and services, net of value-added tax, rebates, discounts, returns and after eliminating intercompany sales. Revenue is recognized when title, risks and rewards of the products are transferred to customers.

### *Revenue from out-licensing*

Out-licensing agreements are concluded with third parties, where the counterparty has to pay license fees. In situations where no further performance commitment exists, revenue is recognized on the earlier of when payments are received or collection is assured. Where continuous involvement for a certain period is required in the form of technology transfer or technical support, revenues are recognized over the period in question.

### *Revenue associated with up-front payments or performance milestones*

Such revenue is recognized in accordance with respective agreements.

### *Revenue from royalties*

Royalty payments are recognized on an accrual basis in accordance with the respective agreements.

### *Interest income*

Interest income is recognized on a pro rata temporis basis using the effective interest method.

## **Development / intangible assets**

Development expenses are charged to the income statement as incurred. They are capitalized as intangible assets when it is probable that future economic benefits will flow to Santhera. Such intangible assets are amortized on a straight-line basis over the period of the expected benefit when the asset becomes available for use, and are reviewed for impairment at each balance sheet date. Assets not available for use are tested annually.

### 3 Critical Accounting Estimates, Assumptions and Judgments

The preparation of financial statements in conformity with IFRS requires the use of certain critical accounting estimates. It also requires management to exercise its judgment in the process of applying Santhera's accounting policies. Santhera makes estimates and assumptions concerning the future. The resulting accounting will not necessarily equal the related actual outcome. The following areas involve assumptions and estimates that can have a significant impact on the consolidated financial statements:

- Measurement and impairment testing of intangible assets, see note 7 "*Impairment Test for Intangible Assets*".
- Measurement and impairment testing of inventory, see note 9 "*Inventories*".
- Personnel expenses from share-based payments in accordance with IFRS 2, i.e. estimates regarding the valuation of equity rights plans when granted, see note 17 "*Equity Rights Plans*".
- Actuarial valuations in the context of defined benefit pension plans where various assumptions on e.g. discount rates, salary increase rates and mortality rates, etc. bear significant uncertainties due to the long-term nature of the plans, see note 21 "*Employee Expenses and Benefits*".

### 4 Exchange Rates of Principal Currencies

	Income statement in CHF average rates		Balance sheet in CHF year-end rates	
	2016	2015	2016	2015
1 Euro (EUR)	1.0902	1.0681	1.0737	1.0826
1 US dollar (USD)	0.9851	0.9624	1.0160	0.9927
1 British pound (GBP)	1.3352	1.4708	1.2498	1.4694
1 Canadian dollar (CAD)	0.7435	0.7534	0.7532	0.7157

## 5 Tangible Assets

	In CHF thousands	Equipment	IT hardware	Leasehold improvements	2016
<b>Cost</b>					
At January 1		225	567	45	837
Additions		17	226	46	289
Disposals		-3	-36	0	-39
Exchange differences		-1	-2	0	-3
Reclassification		-1	1	0	0
<b>At December 31</b>		<b>237</b>	<b>756</b>	<b>91</b>	<b>1,084</b>

<b>Accumulated depreciation and impairment losses</b>					
At January 1		166	231	42	439
Additions		16	150	2	168
Disposals		-3	-36	0	-39
Exchange differences		0	-1	0	-1
<b>At December 31</b>		<b>179</b>	<b>344</b>	<b>44</b>	<b>567</b>
<b>Net book value</b>		<b>58</b>	<b>412</b>	<b>47</b>	<b>517</b>

	In CHF thousands	Equipment	IT hardware	Leasehold improvements	2015
<b>Cost</b>					
At January 1		185	334	42	561
Additions		61	286	3	350
Disposals		-22	-53	0	-75
Exchange differences		1	0	0	1
<b>At December 31</b>		<b>225</b>	<b>567</b>	<b>45</b>	<b>837</b>

<b>Accumulated depreciation and impairment losses</b>					
At January 1		177	211	41	429
Additions		11	73	1	85
Disposals		-22	-53	0	-75
<b>At December 31</b>		<b>166</b>	<b>231</b>	<b>42</b>	<b>439</b>
<b>Net book value</b>		<b>59</b>	<b>336</b>	<b>3</b>	<b>398</b>

## 6 Intangible Assets

	In CHF thousands	Raxone	Fipamezole	IT software/ patents	2016
<b>Cost</b>					
At January 1		30,387	3,918	477	34,782
Additions		0	0	86	86
Disposals		0	0	-28	-28
<b>At December 31</b>		<b>30,387</b>	<b>3,918</b>	<b>535</b>	<b>34,840</b>
<b>Accumulated amortization and impairment losses</b>					
At January 1		1,013	3,918	292	5,223
Additions		3,039	0	57	3,096
Disposals		0	0	-28	-28
<b>At December 31</b>		<b>4,052</b>	<b>3,918</b>	<b>321</b>	<b>8,291</b>
<b>Net book value</b>		<b>26,335</b>	<b>0</b>	<b>214</b>	<b>26,549</b>

	In CHF thousands	Raxone	Fipamezole	IT software/ patents	2015
<b>Cost</b>					
At January 1		30,387	3,918	312	34,617
Additions		0	0	165	165
<b>At December 31</b>		<b>30,387</b>	<b>3,918</b>	<b>477</b>	<b>34,782</b>
<b>Accumulated amortization and impairment losses</b>					
At January 1		26,157	3,918	268	30,343
Additions		1,013	0	24	1,037
Reversal impairment		-26,157	0	0	-26,157
<b>At December 31</b>		<b>1,013</b>	<b>3,918</b>	<b>292</b>	<b>5,223</b>
<b>Net book value</b>		<b>29,374</b>	<b>0</b>	<b>185</b>	<b>29,559</b>

As a result of receiving the European marketing authorization in September 2015, Santhera determined the recoverable amount of its previously impaired intangible asset "Raxone". This resulted in a reversal of impairment of CHF 26.2 million which has been recorded under Development expenses (see note 7 "Impairment Test for Intangible Assets").

## 7 Impairment Test for Intangible Assets

During 2016 there was no trigger for impairment of intangible assets. "Raxone" represents the main intangible asset of Santhera. It has become available for use in September 2015 and has an estimated useful life of 10 years.

Prior to that it was not available for use and did not generate cash inflows. It was subject to annual impairment testing and had previously been impaired in 2012. Based on the European marketing authorization, received in September 2015, an impairment test was performed at the time which resulted in the full reversal of the previous impairment and an increase in the carrying amount of the intangible asset to its recoverable amount of CHF 29.4 million. At the same time the intangible asset formerly not available for use was transformed into an asset available for use with a finite useful life of 10 years. Amortization of the asset began in September 2015.

Management used the risk-adjusted Net Present Value (**rNPV**) model taking into consideration the expected cumulative probability of reaching the market to calculate recoverable amount. This is a customary model for the valuation of pharmaceutical intangibles. The rNPV model considers the net cash flows over the expected lifetime of the products based on the lifetime of the underlying intellectual property or the market exclusivity granted through orphan drug protection. For the purpose of estimating these cash flows, Santhera made estimates about the expected revenues based on estimated market size and patient numbers, expected market penetration rates, product pricing and project- or product-related costs. Santhera's strategic focus is on LHON and DMD. Since LHON is the most advanced program with a market authorization in the EU, received in 2015, the impairment test for 2015 was entirely based on projected cash flows derived from this program in Europe.

The key assumptions for the tests were as follows:

	<b>2015</b>
Discount rate (WACC)	15%
Market growth rate (terminal value)	0%
Probability of reaching market	100%
Period of projected cash flows	5 years

## 8 Prepaid Expenses and Other Assets

	In CHF thousands	<b>2016</b>	<b>2015</b>
Prepayments		487	1,467
Other assets		96	46
<b>Total at December 31</b>		<b>583</b>	<b>1,513</b>

## 9 Inventories

	In CHF thousands	2016	2015
Raw material (active pharmaceutical ingredients)		5,052	1,552
Semi-finished goods		2,369	1,551
Finished goods		255	338
<b>Total at December 31</b>		<b>7,676</b>	<b>3,441</b>

## 10 Trade and Other Receivables

	In CHF thousands	2016	2015
Trade receivables		3,412	1,466
Other receivables		864	665
<b>Total at December 31</b>		<b>4,276</b>	<b>2,131</b>

Trade receivables in 2016 result from product sales, see note 18 *"Segment and Geographic Information"*. Other receivables consist mainly of amounts due from the government for tax reimbursements (e.g. VAT). They are due within 30 to 120 days and bear no interest. No allowance for doubtful debts was recognized on the receivables as management estimates that no allowance is necessary as of December 31, 2016, and 2015.

## 11 Cash and Cash Equivalents

	In CHF thousands	2016	2015
Cash at banks and on hand			
In CHF		44,358	69,570
In EUR		4,661	6,270
In GBP		546	772
In USD		149	191
In CAD		67	56
Other currencies		34	0
<b>Total at December 31</b>		<b>49,815</b>	<b>76,859</b>

Of which: Short-term money market deposits

In CHF		31,000	45,000
--------	--	--------	--------

## 12 Share Capital

### Ordinary share capital

As of January 1, 2015, the share capital amounted to CHF 4,974,492, divided into 4,974,492 shares ("Shares") at a nominal value of CHF 1 each. During 2015, 398,306 Shares were issued from conditional capital upon the exercise of stock options. 590,000 Shares were issued from authorized capital for a private placement (accelerated bookbuilding) and 300,000 Shares were issued from conditional capital for sale by an independent broker. As a result, as of December 31, 2015, the share capital amounted to CHF 6,262,798, divided into 6,262,798 Shares at a nominal value of CHF 1 each.

During 2016, 17,059 Shares were issued from conditional capital upon the exercise of stock options. As a result, as of December 31, 2016, the share capital amounted to CHF 6,279,857, divided into 6,279,857 Shares at a nominal value of CHF 1 each.

### *Treasury shares*

In connection with the liquidation of Oy Juvantia Pharma, Turku, Finland (**Juvantia**), a company acquired in 2009, Santhera received 8,028 Shares from former Juvantia shareholders. These treasury shares served as pledge from the former owners of Juvantia for compensation of a potential tax claim related to pre-acquisition activities. The claim was resolved and the Shares were sold with a financial profit of TCHF 186.

In the second half of 2016, Santhera entered into an agreement for market making with a well-known bank. Independently, the bank buys and sells Shares on the market on behalf of the Company. On December 31, 2016, Santhera held 3,616 treasury Shares.

### Authorized share capital

On the occasion of the ASM on May 11, 2016, the shareholder approved the increase of the authorized share capital as well as an extension. The Board is authorized to increase the share capital at any time until May 10, 2018, through the issuance of up to 1,500,000 Shares with a nominal value of CHF 1 each. An increase in instalments is permitted. For each such increase, the Board has to determine the issue price, the type of payment, the date of issuance of new Shares, the conditions for the exercise of pre-emptive rights and the beginning date for dividend entitlement.

### Conditional share capital

At the ASM on May 11, 2016, the shareholders additionally approved a maximum increase of the share capital by an aggregate amount of CHF 550,000 (2015: CHF 800,000) through the issuance of a maximum of 550,000 (2015: 800,000) Shares with a nominal value of CHF 1 each. The Shares can be issued through the exercise of equity rights which are granted according to respective regulations of the Board.

There was no change to the maximum increase in conditional share capital which amounted to CHF 650,000 (2015: CHF 650,000) through the issuance of a maximum of 650,000 (2015: 650,000) Shares with a nominal value of CHF 1 per Share by the exercise of option and/or conversion rights which can be granted in connection with the issuance of bonds, similar obligations or other financial instruments by the Company or another Group company, and/or by the exercise of options which are granted by the Company or another Group company. In the case of the issue of bonds, similar obligations or other financial instruments linked with option and/or conversion rights, and in the case of the issue of option rights, the pre-emptive right of shareholders is excluded.

As of December 31, 2016, the Company had a conditional share capital, pursuant to the above provisions, whereby the share capital may be increased by

- a maximum amount of CHF 532,941 (2015: CHF 401,694) through the issuance of up to 532,941 (2015: 401,694) Shares, under the exclusion of shareholders' pre-emptive rights, for option rights being exercised under the Company's stock option plans, see note 17 *"Equity Rights Plans"*, and
- a maximum amount of CHF 650,000 (2015: CHF 650,000) by issuing up to 650,000 (2015: 650,000) Shares, through the exercise of warrants/options and/or notes granted in connection with bonds or similar debt instruments linked with option and/or conversion rights granted by the Company.

## 13 Deferred Taxes

### Net deferred taxes recorded

	In CHF thousands	2016	2015
Temporary differences on inventory		1,067	3,061
Temporary difference on accruals		39	0
<b>Deferred tax assets recognized</b>		<b>1,106</b>	<b>3,061</b>
Temporary differences on intangible assets		2,154	5,167
Temporary differences on intercompany loans		13,449	0
Tax loss carryforwards		-15,603	-5,167
<b>Deferred tax liabilities recognized</b>		<b>0</b>	<b>0</b>
Tax loss carryforwards		301,667	269,696
Of which recorded		-195,583	-25,834
<b>Of which unrecorded</b>		<b>106,084</b>	<b>243,862</b>
Expiring in			
1 year		5,832	9,738
2 years		22,671	5,832
3 years		27,366	22,671
4 years		4,223	177,282
5 years		0	0
More than 5 years		17,942	0
Without expiration		28,050	28,339
<b>Total unrecorded tax loss carryforwards</b>		<b>106,084</b>	<b>243,862</b>

Due to the uncertainty surrounding the future results of operations and the uncertainty as to whether Santhera can use the loss carryforwards for tax purposes, deferred tax assets on tax loss carryforwards were only considered to the extent that they offset taxable temporary differences within the same taxable entity. As there are no temporary differences associated with investments in subsidiaries, no deferred tax liability has to be recognized. No deferred tax assets are calculated on temporary differences related to pension obligations from IAS 19 (TCHF 6,183 per December 31, 2016, and TCHF 3,957 per December 31, 2015, respectively).

## 14 Trade and Other Payables

	In CHF thousands	2016	2015
Trade payables		3,574	3,290
Other payables (nonfinancial)		884	376
<b>Total at December 31</b>		<b>4,458</b>	<b>3,666</b>

All positions are noninterest-bearing and usually settled within 30 to 60 days.

## 15 Accrued Expenses

	In CHF thousands	2016	2015
Development programs		749	699
Liabilities to employees		2,013	905
Accruals for pricing and reimbursement		1,107	673
Accrued marketing and sales expenses		953	629
Expenses for audit, consulting and other		696	331
Accruals for income taxes		282	45
<b>Total at December 31</b>		<b>5,800</b>	<b>3,282</b>

## 16 Commitments and Contingent Liabilities

### Commitments

#### *Commitment for operating lease (noncancellable)*

	In CHF thousands	2016	2015
Within 1 year		734	398
After 1 year through to 5 years		804	278
After 5 years		0	15
<b>Total at December 31</b>		<b>1,538</b>	<b>691</b>

## Contingent liabilities

### *Collaboration and license agreement with Takeda*

In September 2013, Santhera announced an agreement with Takeda Pharmaceutical Company Ltd, Osaka, Japan (**Takeda**) to license back all previously granted rights in DMD and Friedreich's ataxia (**FA**) in order to increase its strategic flexibility. In return, Takeda is eligible to obtain a percentage from future licensing and/or sales income generated by Santhera in DMD of up to EUR 7.0 million. In addition, Santhera has obtained the right to cross-reference Takeda's *idebenone* data for regulatory use in any indication and in any territory. If Santhera makes use of such cross-reference right, Takeda is eligible to obtain a percentage from future licensing and/or sales income generated by Santhera in such indications of up to EUR 3.0 million. Lastly, both companies agreed to terminate a similar agreement for FA signed in 2005 and Santhera's contingent liability of EUR 1.0 million payable to Takeda has been waived. Takeda is eligible to receive up to EUR 1.0 million as a percentage from future income generated by Santhera to offset this waiver.

### *Agreement with the University of Leuven*

In March 2005, Santhera entered into an agreement with Katholieke Universiteit Leuven, Leuven, Belgium (**KU Leuven**), under which KU Leuven assigned to Santhera its patents and patent applications relating to the use of Raxone to treat various forms of muscular-dystrophy-related disorders, particularly DMD. Based on this agreement, Santhera has filed patent applications in major territories covering the use of Raxone for the treatment of DMD.

KU Leuven is entitled to a success fee of up to EUR 0.4 million if and when Santhera commercializes any product in a major market, which includes the EU, the US or Japan and certain countries within the EU. In addition, in the event Santhera commercializes the product itself, KU Leuven is entitled to receive 5% royalties on net sales. In the event Santhera grants commercialization rights to a third party, KU Leuven will receive 15% of all the consideration received by Santhera from such third party.

### *License agreement with Novartis*

On June 30, 2007, Santhera entered into an agreement with Novartis Pharma AG, Basel, Switzerland (**Novartis**), under which it in-licensed *omigapil*. Santhera develops *omigapil* for the treatment of congenital muscular dystrophy (**CMD**). Additional payments will be due to Novartis a) upon start of a pivotal clinical trial, b) upon regulatory approval in a major market country, and c) after reaching certain commercialization milestones. Santhera will also have to pay royalties to Novartis calculated on net sales.

### *Agreement with the National Institutes of Health*

In June 2013, Santhera has obtained an exclusive license from the National Institutes of Health, Bethesda/Maryland, US (**NIH**), to its rights on a patent granted in the US for the use of *idebenone* for the treatment of primary progressive multiple sclerosis (**PPMS**). Under the terms of the agreement, Santhera would have to make certain milestone payments to the NIH not exceeding USD 1.4 million in total. Furthermore, the NIH is eligible to a royalty fee of 3% on net sales and 15% of considerations received in case Santhera sublicenses the program.

*Contracts for clinical development and other*

As part of its ordinary course of business, Santhera has entered into several contracts for e.g. clinical or technical development services. Commitments are within current market prices and can be terminated at the Company's discretion.

In order to meet its requirements for market supply, potential launch and inventory risk management purposes (security stock), Santhera entered into commitments for the purchase of active pharmaceutical ingredients in the amount of up to EUR 2.6 million (to be delivered in 2017).

**Contingent liabilities summary**

Santhera believes that the disclosures above and accruals (see note 15 "Accrued Expenses") are adequate based upon currently available information. However, given the inherent difficulties in estimating liabilities relating to clinical development, regulatory, tax, possible litigation and certain other matters due to uncertainty concerning both the amount and timing of future expenditures, it cannot be guaranteed that additional costs will not be incurred materially beyond the amounts accrued.

**17 Equity Rights Plans**

Santhera has established equity rights plans to align the long-term interests of the members of the Board, the Executive Management and employees. Rights granted under these plans are equity-settled.

**17.1 Stock Option Plans****Executive Incentive Plan (EIP)**

In November 2006, under the EIP, the members of the Executive Management were granted stock options to acquire 101,065 Shares, as a management incentive. Each of these stock options entitles its holder to purchase one Share at an exercise price of CHF 1. The vesting period of the options was one year. At the end of the option term, i.e. after a period of ten years as from the grant date, all unexercised stock options will expire without value. The EIP is administered under the responsibility of the Board. No further grants can be made under the EIP.

*Options outstanding, exercised or forfeited under the EIP*

Number of options Plan	2016				2015			
	Exercised	For- feited	Expired	Outstand- ing	Exercised	For- feited	Expired	Out- standing
EIP	1,210	0	0	0	790	0	0	1,210

## Employee Stock Option Plans

The Company adopted the ESOP 2004, ESOP 2008, ESOP 2010 and ESOP 2015 (collectively the **ESOPs**) to provide incentives to the Executive Management, employees and consultants helping to ensure their commitment to Santhera over the long-term. Since January 1, 2015, new grants have been allocated under the ESOP 2015. Option grants are made periodically at the discretion of the Board or as contractually agreed with employees. The ESOPs contain customary provisions in respect of the adjustment or cancellation of stock options upon termination of employment, retirement, death, disability and certain corporate transactions. All stock option plans are administered under the responsibility of the Board. Each stock option entitles its holder to purchase one Share of the Company at an exercise price defined to be either a) equal to the volume-weighted average share price in the three preceding months for Swiss employees, or b) the closing share price on the SIX Swiss Exchange (**SIX**) at each grant date. In general, 50% of the stock options vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. At the end of the option term, i.e. after a period of 10 years as from the grant date, unexercised stock options expire without value. Subject to the provisions of the ESOP 2004, vested stock options of employees leaving the Company in good faith do not lapse. Under the ESOP 2008 and ESOP 2010 vested stock options of employees leaving the Company in good faith expire six months after the termination date of the employment. Under the ESOP 2015 vested stock options of employees leaving the Company in good faith do not expire. Unvested stock options of employees leaving the Company are forfeited under all stock option plans.

### *Options outstanding, exercised, forfeited or expired under ESOPs*

Number of options						<b>2016</b>
	At January 1	Exercised	Granted	Forfeited	Expired	At December 31
ESOP 2004	26,091	-4,825	0	0	-20,513	753
ESOP 2008	1,500	-1,500	0	0	0	0
ESOP 2010	47,773	-9,524	0	0	0	38,249
ESOP 2015	140,260	0	135,830	-15,289	0	260,801
<b>Total</b>	<b>215,624</b>	<b>-15,849</b>	<b>135,830</b>	<b>-15,289</b>	<b>-20,513</b>	<b>299,803</b>

Number of options						<b>2015</b>
	At January 1	Exercised	Granted	Forfeited	Expired	At December 31
ESOP 2004	35,136	-9,045	0	0	0	26,091
ESOP 2008	1,500	0	0	0	0	1,500
ESOP 2010	409,444	-358,971	0	-2,700	0	47,773
ESOP 2015	0	0	142,260	-2,000	0	140,260
<b>Total</b>	<b>446,080</b>	<b>-368,016</b>	<b>142,260</b>	<b>-4,700</b>	<b>0</b>	<b>215,624</b>

## Board Stock Option Plans

The Company adopted the BSOP 2011 and BSOP 2015 (collectively the **BSOPs**) to provide incentives to members of the Board. Since January 1, 2015, new grants have been made under the BSOP 2015. The plan contains the same customary provisions as under the ESOP plans described above. Each stock option entitles its holder to purchase one Share of the Company at an exercise price defined to be either a) equal to the volume-weighted average share price in the three preceding months, or b) the closing share price on the SIX at each grant date. In general, 50% of the stock options vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. At the end of the option term, i.e. after a period of 10 years as from the grant date, unexercised stock options expire without value. Under the BSOP 2011 vested stock options of Board members leaving the Board in good faith expire six months after the termination date of them being a member of the Board while unvested stock options of Board members leaving the Board in good faith are forfeited. Under the BSOP 2015 vested and unvested stock options of Board members leaving the Board in good faith do not expire.

### *Options outstanding, exercised, forfeited or expired under BSOPs*

Number of options	<b>2016</b>				
	Exercised	Granted	Forfeited	Expired	Outstanding
BSOP 2015	0	6,562	0	0	13,562
<b>Total</b>	<b>0</b>	<b>6,562</b>	<b>0</b>	<b>0</b>	<b>13,562</b>

Number of options	<b>2015</b>				
	Exercised	Granted	Forfeited	Expired	Outstanding
BSOP 2011	29,500	0	0	0	0
BSOP 2015	0	7,000	0	0	7,000
<b>Total</b>	<b>29,500</b>	<b>7,000</b>	<b>0</b>	<b>0</b>	<b>7,000</b>

Since July 1, 2016, no more stock options (December 31, 2015: 177,860) are available for future grants under the ESOP 2015 and/or the BSOP 2015. Stock options are replaced by Share Appreciation Rights (**SAR**), see note 17.2 *"Share Appreciation Rights Plans"*.

### Fair value calculations for stock options granted

The fair value of stock options is determined at each grant date by using the Hull-White pricing model. The calculation of the option value was performed by applying the following parameters:

	2016	2015
Market price of stock	CHF 37.05 to 91.25	CHF 80.20 to 138.90
Exercise prices	CHF 69.30 to 89.45	CHF 83.00 to 133.08
Weighted average fair value of options granted	CHF 24.18	CHF 40.12
Expected volatility <sup>1</sup>	38% to 39%	43% to 46%
CHF risk-free interest rate	0.0% p.a.	-0.10% to 0.38% p.a.
Option term <sup>2</sup>	10 years	10 years
Expected dividend yield	0%	0%

<sup>1</sup> The expected volatility was determined on the basis of selected biotech companies.

<sup>2</sup> After expiration of the vesting period, the stock options become American-style options and may be exercised any time until the end of the option term. The option-pricing model takes into consideration certain assumptions about potential early exercises.

### Number of stock options outstanding and exercisable

	Number of options	2016	2015
<b>Outstanding at January 1</b>		<b>223,834</b>	<b>477,580</b>
Granted		142,392	149,260
Exercised <sup>1</sup>		-17,059	-398,306
Forfeited		-15,289	-4,700
Expired		-20,513	0
<b>Outstanding at December 31</b>		<b>313,365</b>	<b>223,834</b>
<b>Exercisable at December 31</b>		<b>36,327</b>	<b>60,412</b>

<sup>1</sup> The average closing share price of options exercised during the reporting period 2016 was CHF 68.12 (2015: CHF 95.40).

The value of stock options granted is recognized as personnel expense over the period Santhera receives services. In 2016, stock option grants resulted in personnel expenses of TCHF 3,311 (TCHF 418 related to Development, TCHF 1,766 related to Marketing and sales (M&S) and TCHF 1,127 to General and administration (G&A)) and in 2015, such grants resulted in personnel expenses of TCHF 1,528 (TCHF 277 related to Development, TCHF 580 related to M&S and TCHF 671 to G&A).

**Terms of options outstanding at December 31**

Exercise price range for options (in CHF)	Number outstanding	Weighted average remaining contractual life (years)	2016 Number exercisable	Number outstanding	Weighted average remaining contractual life (years)	2015 Number exercisable
1.00	0	0	0	1,210	0.86	1,210
from 3.78 to 6.34	33,699	6.66	33,574	42,673	7.44	31,611
from 22.25 to 30.10	4,550	7.48	2,000	6,600	7.43	1,500
from 59.44 to 69.30	18,800	9.23	0	19,788	0.52	19,788
from 82.10 to 114.50	256,316	8.61	753	153,563	9.02	6,303
<b>Total</b>	<b>313,365</b>	<b>8.42</b>	<b>36,327</b>	<b>223,834</b>	<b>7.87</b>	<b>60,412</b>

**17.2 Share Appreciation Rights Plans**

Starting with July 1, 2016, Santhera switched from stock option plans to Share Appreciation Rights Plans (**SARP**). It introduced a Board Share Appreciation Rights Plan (**BSARP 2016**) for the members of its Board and an Employee Share Appreciation Rights Plan (**ESARP 2016**) for the Executive Management, employees and consultants. SAR grants are made periodically at the discretion of the Board or as contractually agreed with employees. The SARPs contain customary provisions in respect of the adjustment or cancellation of SARs upon termination of employment, retirement, death, disability and certain corporate transactions. All SARPs are administered under the responsibility of the Board. In general, 50% of the SARs vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. At the end of the SAR term, i.e. after a period of 10 years as from the grant date, unexercised SARs expire without value. Upon exercise of one SAR, participants receive the difference between the price of one Share at the time of exercise and the base value ("exercise price" as defined upon grant), in Shares. Subsequently, participants may sell their Shares.

*SARs outstanding, exercised, forfeited or expired under SARP*

Number of SARs	2016				
	Exercised	Granted	Forfeited	Expired	Outstanding
ESARP 2016	0	56,581	0	0	56,581

SARP were adopted since July 1, 2016, only.

### Fair value calculations for SARs granted

The fair value of SARs is determined at each grant date by using the Hull-White pricing model. The calculation of the SAR value was performed by applying the following parameters:

	<b>2016</b>
Market price of stock	CHF 37.05 to 91.25
Exercise prices	CHF 51.75 to 76.50
Weighted average fair value of SARs granted	CHF 22.12
Expected volatility <sup>1</sup>	38% to 39%
CHF risk-free interest rate	0.0% p.a.
SAR term <sup>2</sup>	10 years
Expected dividend yield	0%

<sup>1</sup> The expected volatility was determined on the basis of selected biotech companies.

<sup>2</sup> After expiration of the vesting period, the SARs become rights similar to American-style options and may be exercised any time until the end of the SAR term. The SAR pricing model takes into consideration certain assumptions about potential early exercises.

### Number of SARs outstanding and exercisable

	Number of options	<b>2016</b>
<b>Outstanding at January 1</b>		<b>0</b>
Granted		56,581
Exercised		0
Forfeited		0
Expired		0
<b>Outstanding at December 31</b>		<b>56,581</b>
<b>Exercisable at December 31</b>		<b>0</b>

The value of SARs granted is recognized as personnel expense over the period Santhera receives services. In 2016, SAR grants resulted in personnel expenses of TCHF 150 (TCHF 15 related to Development, TCHF 135 related to M&S and TCHF 0 to G&A) and in 2015, no SARs were granted.

Santhera plans to conditionally allocate up to 198,162 SARs in the first quarter of 2017. These SARs form part of the long-term incentive (LTI) award to employees for the year ended December 31, 2016. Although these SARs were not legally granted in 2016, Executive Management considers it appropriate to recognize expenses in 2016 as employees have been rendering services in 2016 in expectation of the annual LTI allocation. Personnel expenses in 2016 for this amounted to TCHF 1,222 (TCHF 332 related to Development, TCHF 569 related to M&S and TCHF 321 related to G&A) based on an estimate of fair value. The allocation of these SARs becomes unconditional once the compensation is approved on the occasion of the ASM, to be held on April 4, 2017. After the ASM the grant date fair value of the SARs will be determined and the cumulative expense adjusted.

**Terms of SARs outstanding at December 31**

Exercise price range for SARs (in CHF)	Number outstanding	Weighted average remaining contractual life (years)	2016 Number exercisable
from 51.75 to 76.50	56,581	9.71	0
<b>Total</b>	<b>56,581</b>	<b>9.71</b>	<b>0</b>

**18 Segment and Geographic Information****Segment information**

Santhera operates in one operating segment, the development and commercialization of specialty niche products for the treatment of mitochondrial and neuromuscular diseases. The Board, the Executive Management and senior managers, being the CODM, assess the reporting data and allocate resources as one segment on an aggregated consolidated level according to the operating expenses by function. Santhera generates revenue from sales of Raxone for the treatment of LHON. Geographic revenue information is based on location of the customer.

**Geographic information***Net sales*

	In CHF thousands	2016	2015
EU		19,002	4,321
Rest of the world		31	0
<b>Total</b>		<b>19,033</b>	<b>4,321</b>

In 2016, net sales amounted to CHF 19.0 million. Raxone was sold into 15 EU countries, with the majority of sales reached in France and Germany. In 2015, net sales of Raxone were generated after European marketing authorization in LHON and under special programs (e.g. the French temporary authorization for use as well as international Named Patient Programs) in the amount of CHF 4.3 million.

*Noncurrent assets (excluding financial instruments and deferred taxes)*

	In CHF thousands	2016	2015
Switzerland		26,966	29,876
EU		100	80
North America		0	1
<b>Total</b>		<b>27,066</b>	<b>29,957</b>

## 19 Other Operating Income

This position consists primarily of reimbursements from scientific programs.

## 20 Operating Expenses by Nature

	In CHF thousands	2016	2015
External Development expenses		-12,119	-6,341
Reversal of impairment of intangible asset		0	26,157
Reversal of impairment on inventories		0	947
Patent and license expenses		-280	-222
Marketing expenses		-10,121	-3,870
Employee expenses		-21,403	-13,105
<i>Of which non-cash-relevant expenses for share-based payments</i>		-4,683	-2,040
Other administrative expenses		-3,796	-2,999
Depreciation and amortization		-225	-110
Lease expenses		-587	-406
Other operating expenses		-107	-16
<b>Total operating expenses</b>		<b>-48,638</b>	<b>35</b>

## 21 Employee Expenses and Benefits

### Employee expenses

	In CHF thousands	2016	2015
Wages and salaries		-12,397	-6,435
Social security and other personnel-related expenses <sup>1</sup>		-4,323	-4,630
<i>Of which non-cash-relevant adjustments of pension fund</i>		-450	394
Share-based payments		-4,683	-2,040
<b>Total employee costs</b>		<b>-21,403</b>	<b>-13,105</b>

<b>Average number of full-time equivalents<sup>2</sup></b>	<b>65.1</b>	<b>31.4</b>
<b>Full-time equivalents at year-end</b>	<b>74.4</b>	<b>53.3</b>
<b>Total headcount at year-end</b>	<b>80</b>	<b>59</b>

<sup>1</sup> Thereof TCHF 124 were expensed for defined contribution plans in North America and some EU countries (2015: TCHF 18).

<sup>2</sup> For the calculation of full-time equivalents, only employees with part-time and full-time permanent working contracts are taken into consideration.

## Pension plan

In accordance with the Swiss pension fund law "Federal Act on Occupational Old Age, Survivors' and Invalidity Pension Provision" (**OPA**), all employees of Santhera Pharmaceuticals Holding AG and Santhera Pharmaceuticals (Schweiz) AG, both in Liestal, Switzerland, have to be affiliated with a collective independent pension fund. These funds provide for retirement benefits, as well as risk benefits (death and disability). The plans qualify as defined benefit plans under IAS 19 and the assets cannot revert to the employer. Contributions to the plans are such that the employee contributes 40% and the employer the rest. Contributions are computed as percentage of the salary, depending on age. In order to manage these risks, Santhera entered into an agreement with AXA Foundation for occupational benefits (**AXA foundation**). The AXA foundation is responsible for the governance of the plan; the board is composed of an equal number of representatives from the employers and employees chosen from all affiliated companies. AXA foundation has set up investment guidelines, defining in particular the strategic allocation with margins. AXA foundation has reinsured its risks (investment risk, mortality risk, etc.) with AXA Life Ltd, Winterthur, Switzerland (**AXA**). AXA manages the savings capital/investments on behalf of AXA foundation. The accumulated savings capital is allocated to each insured individual and consists of annual contributions, savings credits and interest credits. In certain situations, additional payments or increased periodic contributions by the employer may become due based on the pension plans funded status as measured under Swiss pension rules (**OPA**).

An independent actuary has performed the respective calculations as required by IAS 19:

### *Changes in defined benefit obligations*

	In CHF thousands	2016	2015
<b>Present value of obligation, January 1</b>		<b>15,797</b>	<b>7,747</b>
Current employer service cost		1,038	704
Past service cost <sup>1</sup>		0	-656
Interest cost		142	76
Employee contributions		382	267
Benefits paid / transfer payments		2,259	6,074
Insurance premiums		-192	-142
Remeasurements <sup>2</sup>		1,853	1,727
<b>Present value of obligation, December 31</b>		<b>21,279</b>	<b>15,797</b>

<sup>1</sup> Decrease of obligation due to reduction of the conversion rates for the over-mandatory part of the retirement capital.

<sup>2</sup> Details of remeasurements:

	In CHF thousands	2016	2015
Effect of changes in demographic assumptions		-435	0
Actuarial (gain)/loss due to changes in financial assumptions		599	170
Actuarial (gain)/loss due to experience adjustments		1,689	1,557
<b>Subtotal (gain)/loss</b>		<b>1,853</b>	<b>1,727</b>
(Return)/loss on plan assets (excluding interest income)		-77	-56
<b>Total remeasurements in other comprehensive income (gain)/loss</b>		<b>1,776</b>	<b>1,671</b>

*Changes in plan assets*

	In CHF thousands	2016	2015
<b>Fair value of assets, January 1</b>		<b>11,840</b>	<b>5,067</b>
Interest income on assets		110	55
Employer contributions		620	463
Employee contributions		382	267
Benefits paid / transfer payments		2,259	6,074
Insurance premiums		-192	-142
Remeasurements (return/(loss) on plan assets (excluding interest income))		77	56
<b>Fair value of assets, December 31</b>		<b>15,096</b>	<b>11,840</b>

*Net defined benefit asset/(obligation)*

	In CHF thousands	2016	2015
Present value of obligation, December 31		21,279	15,797
Fair value of assets, December 31		15,096	11,840
<b>Net defined asset/(obligation)</b>		<b>-6,183</b>	<b>-3,957</b>

*Asset breakdown*

	In CHF thousands		2016		2015	
	Quoted market price	Not quoted market price	Quoted market price	Not quoted market price	Quoted market price	Not quoted market price
Insurance contract	0	15,096	0	11,840		
<b>Total value of assets</b>	<b>0</b>	<b>15,096</b>	<b>0</b>	<b>11,840</b>		

An asset breakdown is not available. The assets of Santhera's defined benefit plan have no quoted market price since AXA fully insures them as an insurance contract.

*The weighted-average assumptions to determine benefit obligations and defined benefit cost were as follows:*

	In %	2016	2015
Discount rate		0.65	0.90
Expected future salary increases		1.50	1.50

*Sensitivity analysis for 2016:*

In CHF thousands	Defined benefit obligation		Gross service cost	
	Increase as- sumption	Decrease assumption	Increase assumption	Decrease assumption
Discount rate +/-0.25%	-896	961	-117	125
Salary increase +0.25%	130	-	-1	-
Live expectancy +1 year	431	-	32	-

*Sensitivity analysis for 2015:*

In CHF thousands	Defined benefit obligation		Gross service cost	
	Increase as- sumption	Decrease assumption	Increase assumption	Decrease assumption
Discount rate +/-0.25%	-537	578	-73	78
Salary increase +0.25%	84	-	-1	-
Live expectancy +1 year	245	-	20	-

*Mortality rate:*

Life expectancy at age 65	<b>2016</b>	<b>2015</b>
Male	22.4	21.6
Female	24.4	24.1

The expected employer contributions for fiscal year 2017 amount to approximately TCHF 876 (2015: TCHF 568). No benefit obligations for pensioners exist at December 31, 2016 (2015: none). The duration of the plan liabilities calculated is 21.6 years as per December 31, 2016 (2015: 20.8 years).

## 22 Financial Income/Expenses

### Financial income

	In CHF thousands	2016	2015
Interests on cash and cash equivalents		5	2
Realized and unrealized foreign exchange gains		923	414
<b>Total</b>		<b>928</b>	<b>416</b>

### Financial expenses

	In CHF thousands	2016	2015
Interest expenses		-15	-11
Realized and unrealized foreign exchange losses		-980	-644
<b>Total</b>		<b>-995</b>	<b>-655</b>

## 23 Income Taxes

	In CHF thousands	2016	2015
Current income tax income/(expense)		-266	-46
Deferred tax income/(expense)		-1,955	3,061
<b>Total</b>		<b>-2,221</b>	<b>3,015</b>

The following is a theoretical reconciliation of tax expense and the accounting profit multiplied by expected income tax rate of principal:

	In CHF thousands	2016	2015
Result before taxes		-33,194	2,934
Tax (expense)/income at expected group tax rate of 9.3% <sup>1</sup>		3,087	-587
Effect of tax rate difference group versus local		-724	-2,413
Effect of non-deductible expenses		-372	-12
Prior year DTA decrease		-249	0
Utilization of previously unrecognized tax losses		20	7,376
Recognition of previously unrecognized DTL		-13,449	0
Recognition of DTA on previously unrecognized tax losses		13,449	4,336
Unrecognized deferred taxes		-3,983	-5,685
<b>Effective tax income/(expense)</b>		<b>-2,221</b>	<b>3,015</b>

<sup>1</sup> In 2016 the principal (Santhera Pharmaceuticals (Schweiz) AG) obtained a tax ruling according to which the expected tax rate is reduced to 9.3% (2015: 20%).

The net effect regarding the change in tax rate is CHF 0 because DTAs and DTLs recognized are in the same amount. According to currently applicable Swiss tax law, the period to offset tax loss carryforwards against taxable profit is limited to seven years. According to currently applicable German tax law, tax loss carryforwards can, besides other conditions, be offset against taxable profit for an unlimited period but only to an amount of EUR 1.0 million and in addition for 60% of further amounts beyond this threshold per annum.

## 24 Earnings/Loss per Share

Basic earnings/loss per share is calculated by dividing the net profit/net loss attributable to equity holders by the weighted average number of Shares issued and outstanding during the reporting period, excluding Shares held as treasury shares (purchased at market).

	<b>2016</b>	<b>2015</b>
Net result attributable to shareholders (in CHF)	-35,414,845	5,949,239
Weighted average number of shares issued and outstanding	6,273,460	5,343,089
<b>Basic net result per share (in CHF)</b>	<b>-5.65</b>	<b>1.11</b>

Diluted earnings per share are calculated by dividing the net profit attributable to owners of ordinary Shares of the Company by the weighted average number of Shares issued and outstanding during the reporting period adjusted for Shares held as treasury shares (purchased at market) and the number of potential shares from stock option plans. For 2016, no diluted net result was calculated since the exercise of stock options would have been anti-dilutive.

	<b>2016</b>	<b>2015</b>
Net result attributable to shareholders (in CHF)	-35,414,845	5,949,239
Weighted average number of shares issued and outstanding	6,273,460	5,343,089
Additional shares of potential option exercise	0	140,441
Adjusted weighted average number of shares issued and outstanding	6,273,460	5,483,530
<b>Diluted net result per share (in CHF)</b>	<b>-5.65</b>	<b>1.08</b>

## 25 Related Party Transactions

### Board and Executive Management compensation

#### *Total compensation of Board and Executive Management*

	In CHF thousands	<b>2016</b>	<b>2015</b>
Compensation, wages and salaries		2,546	2,043
Post-employment benefits (pension fund contributions)		221	211
Share-based payment expenses (fair value according to IFRS 2)		1,508	855

### *Transactions with members of the Board and Executive Management*

There are no loans outstanding or guarantee commitments granted to members of the Board and Executive Management.

In 2016, no stock options were exercised by members of the Board (2015: 29,500 stock options exercised). During 2016, 3,999 stock options were exercised by the Executive Management (2015: 211,394 stock options exercised).

## **26 Risk Management Objectives and Policies**

Santhera Pharmaceuticals Holding AG maintains a Group-wide corporate risk management system consisting of the areas corporate governance, financial internal controls and quality control / quality assurance.

On a regular basis, operational corporate risks are identified and their likelihood and impact assessed (gross risks). By defining and undertaking appropriate measures, these risks are managed accordingly to either reduce or avoid such risk (net risk). The results of this process are discussed at Board meetings.

Those risks as identified within the area of accounting and financial reporting as well as related control processes are further covered by the Company's Group-wide internal control system.

Santhera conducts development activities primarily in Switzerland, the EU and the US and is exposed to a variety of financial risks, such as, but not limited to, foreign exchange rate risk, credit risk, liquidity risk, cash flow and interest rate risk. Part of Santhera's overall risk management focuses on financial risks and the unpredictability of financial markets seeking to minimize potential adverse effects on the financial performance of the Group. Special guidelines and policies approved by the Board exist for overall risk management, financial internal controls and treasury management and are monitored by the Executive Management and the Board on a regular basis. The risk of foreign exchange rate fluctuations on the expenses can partly be managed by entering into foreign exchange derivative contracts. In accordance with the relevant treasury guidelines, Santhera only concludes contracts with selected high-quality financial institutions of good reputation and is not allowed to engage in speculative transactions. In addition, Santhera's treasury guidelines currently limit the Company to engage in money market deposits or similar instruments with a maturity beyond 12 months.

### **Foreign exchange rate risk**

Santhera holds cash amounts in five major currencies CHF, EUR, USD, GBP and CAD to cover the majority of future expected expenses. In addition, in order to reduce its foreign exchange rate exposure, Santhera occasionally enters into derivative currency contracts (forwards, options, structured derivatives) to hedge against additional major foreign currency exchange rate fluctuations. Evaluations based on market values are performed regularly. Any fair value changes of such currency positions are recorded accordingly in the income statement. Santhera's primary exposure to financial risk is due to fluctuation of exchange rates between CHF, EUR, USD, GBP and CAD. No derivative currency contracts are outstanding as of December 31, 2016 and 2015.

The following table demonstrates the sensitivity to a reasonable possible change in the EUR exchange rate, with all other variables held constant, of the Group's result before taxes. There is no impact on the Group's equity.

	Increase/decrease foreign currency rate	Effect on result before taxes in CHF thousands
<b>EUR positions</b>		
<b>2016</b>	+5%	+187
	-5%	-187
<b>2015</b>	+10%	+608
	-10%	-608

### Interest rate risk

Santhera earns interest income on cash and cash equivalents and its profit and loss may be influenced by changes in market interest rates. Santhera holds its cash on deposit/current accounts or invests cash through money market instruments in line with its treasury guidelines to follow its financial needs over time.

The following table demonstrates the sensitivity to a reasonable change in interest rates, with all other variables held constant, of the Group's result before taxes. There is no impact on the Group's equity.

As per end of 2016, variances of +/-50 basis points were calculated, resulting in fluctuations of +/- TCHF 249 before tax (end of 2015: +/-50 basis points resulting in fluctuations of +/-TCHF 384 before tax).

### Credit risk

Santhera has a certain concentration of credit risk. Short-term investments are invested as cash on deposit or in low-risk money market funds, i.e. money market accounts with government-backed corporate banks, top-tier categorized banks or S&P A-1 rated money market investment instruments or similar ratings. No investment or contract with any single counterparty, except cash on deposit subject to the criteria above, comprises more than 20% of cash and cash equivalents at the date of investment.

Santhera has policies in place to ensure that sales of products or entered partnerships are made to or entered with customers or partners with an appropriate credit history and a commitment to ethical business practices. The maximum credit risk exposure is limited to the carrying amount of its financial assets including derivatives.

### Liquidity risk

Prudent liquidity risk management implies maintaining sufficient cash and cash equivalents. Currently, the Company is financed through equity and convertible bonds (see note 27 "*Events After the Reporting Date*") and there is no interest-bearing funding through debt instruments. Santhera's treasury calculates on a rolling basis the needs for aligning the current expenses against the need for optimized financial investments.

*Contractual undiscounted cash flows*

<b>Year ended December 31, 2016</b> In CHF thousands	On demand	Less than 3 months	3 to 12 months	1 to 5 years	After 5 years	Total	Book value
Accrued expenses	0	3,787	0	0	0	3,787	3,787
Trade payables	0	3,574	0	0	0	3,574	3,574
<b>Total</b>	<b>0</b>	<b>7,361</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>7,361</b>	<b>7,361</b>

<b>Year ended December 31, 2015</b> In CHF thousands	On demand	Less than 3 months	3 to 12 months	1 to 5 years	After 5 years	Total	Book value
Accrued expenses	0	2,377	0	0	0	2,377	2,377
Trade payables	0	3,290	0	0	0	3,290	3,290
<b>Total</b>	<b>0</b>	<b>5,667</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>5,667</b>	<b>5,667</b>

**Categories of financial instruments**

<b>Year ended December 31, 2016</b> In CHF thousands	Book value	Loans and receivables	Other liabilities at amortized cost
<b>Assets</b>			
Financial assets long-term	270	270	0
Trade receivables	3,412	3,412	0
Other receivables	55	55	0
Cash and cash equivalents	49,815	49,815	0
<b>Total</b>	<b>53,552</b>	<b>53,552</b>	<b>0</b>
<b>Liabilities</b>			
Accrued expenses	3,787	0	3,787
Trade payables	3,574	0	3,574
<b>Total</b>	<b>7,361</b>	<b>0</b>	<b>7,361</b>

Year ended December 31, 2015 In CHF thousands	Book value	Loans and receivables	Other liabilities at amortized cost
<b>Assets</b>			
Financial assets long-term	190	190	0
Trade receivables	1,466	1,466	0
Other receivables	49	49	0
Cash and cash equivalents	76,859	76,859	0
<b>Total</b>	<b>78,564</b>	<b>78,564</b>	<b>0</b>
<b>Liabilities</b>			
Accrued expenses	2,377	0	2,377
Trade payables	3,290	0	3,290
<b>Total</b>	<b>5,667</b>	<b>0</b>	<b>5,667</b>

### Capital management

The first priority of Santhera's capital management is to provide adequate cash funds to ensure the financing of successful development and marketing activities so that future profits can be generated by gaining marketing authorization approvals for pharmaceutical products. As a company with currently one product on a smaller market, the capital management continues to be focused on the cash and cash equivalents position and is governed by specific Group treasury guidelines.

The funds raised in various private financing rounds, private placements in 2008, 2014 and 2015, SEDA, the sale of Shares by an independent broker as well as funds generated through product sales and revenue from licensing enabled the Group to be adequately financed.

No changes in goals and policies of the treasury management have been made during the past two reporting years.

## 27 Events After the Reporting Date

On January 25, 2017, Santhera announced that it has received from Ernesto Bertarelli, Donata Guichard-Bertarelli, Maria-Iris Bertarelli, (together, the "Bertarelli Group") and Ralf Arnold, Markus Kühnle and Thomas Terhorst (together, the "Iglu Group") the notification that they have combined their respective shareholdings in Santhera to form a new shareholder group. The above mentioned shareholders have announced that they have formed a new shareholder group with a combined holding in Santhera of 18.84% (1,179,977 shares).

On February 10, 2017, Santhera has successfully placed CHF 60 million senior unsecured convertible bonds (the "Convertible Bonds") due 2022. The Company intends to use the net proceeds from this placement primarily to fund the commercialization of Raxone in the currently approved indication, to prepare the market entry and commercial launch in subsequent indications, for investment into further clinical trials with Raxone and for other corporate purposes.

The Convertible Bonds have a 5-year maturity and a coupon of 5.00% per annum. The conversion price was fixed at CHF 86.4006, representing a premium of 20% over the volume weighted average price (**VWAP**) of the Shares between the launch and pricing of the Convertible Bonds (the "Reference Share Price"). The Convertible Bonds are issued at 100% of their principal amount and, unless previously redeemed, converted or repurchased and cancelled, will mature on February 17, 2022, at 100% of their principal amount.

The Conversion Price will be reset after the first year if the VWAP of the shares during a specified period of time will be below the Reference Share Price. The new Conversion Price must not be lower than 75% of the Conversion Price at issuance. In addition, Santhera may call the Convertible Bonds at any time on or after the second anniversary of the issue date at par, plus accrued interest, if any, if the VWAP of the shares is at least 160% of the Conversion Price.

The Convertible Bonds will be convertible into 694,440 registered Shares of Santhera Pharmaceuticals Holding AG, representing 11.1% of the current outstanding share capital of Santhera Pharmaceuticals Holding AG. The Shares to be delivered upon conversion shall be sourced from conditional and, if needed, from authorized capital.

The Convertible Bonds are listed and traded on the SIX Swiss Exchange since February 16, 2017.

Santhera agreed to a company lock-up ending 90 days after that date, subject to customary exceptions.

# Statutory Auditor's Report on the Audit of the Consolidated Financial Statements

Basle, 6 March 2017

## Opinion

We have audited the consolidated financial statements of Santhera Pharmaceuticals Holding AG and its subsidiaries (the Group), which comprise the consolidated balance sheet as at 31 December 2016 and the consolidated income statement, the consolidated statement of comprehensive income, consolidated cash flow statement and the consolidated statement of changes in equity for the year then ended, and notes to the consolidated financial statements, including a summary of significant accounting policies.

In our opinion the consolidated financial statements (pages 18 to 58) give a true and fair view of the consolidated financial position of the Group as at 31 December 2016, and its consolidated financial performance and its consolidated cash flows for the year then ended in accordance with International Financial Reporting Standards (IFRS) and comply with Swiss law.

## Basis for opinion

We conducted our audit in accordance with Swiss law, International Standards on Auditing (ISAs) and Swiss Auditing Standards. Our responsibilities under those provisions and standards are further described in the Auditor's Responsibilities for the Audit of the Consolidated Financial Statements section of our report.

We are independent of the Group in accordance with the provisions of Swiss law and the requirements of the Swiss audit profession, as well as the IESBA Code of Ethics for Professional Accountants, and we have fulfilled our other ethical responsibilities in accordance with these requirements.

We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our opinion.

## Key audit matters

Key audit matters are those matters that, in our professional judgment, were of most significance in our audit of the consolidated financial statements of the current period. These matters were addressed in the context of our audit of the consolidated financial statements as a whole, and in forming our opinion thereon, and we do not provide a separate opinion on these matters. For each matter below, our description of how our audit addressed the matter is provided in that context.

We have fulfilled the responsibilities described in the *Auditor's responsibilities for the audit of the consolidated financial statements* section of our report, including in relation to these matters. Accordingly, our audit included the performance of procedures designed to respond to our assessment of the risks of material misstatement of the consolidated financial statements. The results of our audit procedures, including the procedures performed to address the matters below, provide the basis for our audit opinion on the consolidated financial statements.

**Revenue recognition related to the measurement of revenue to be recognized for sales to pharmacies and distributors**

<b>Area of focus</b>	<p>The Group enters into contractual arrangements with pharmacies and distributors in different jurisdictions that require the Group to provide rebates and discounts that result in deductions to gross sales and which for unsettled amounts are recognised as an accrual. We focused on this area due to the complexity of jurisdictional laws and regulations governing the determination of the sales price. Specifically, the laws and regulations vary across the Group's markets and establishing appropriate accruals for unsettled amounts may require judgment and estimation.</p> <p><i>See note 2 to these consolidated financial statements for Santhera Pharmaceuticals Holding Ltd's description of the accounting policy for revenue recognition.</i></p>
<b>Our audit response</b>	<p>Our audit procedures included gaining an understanding of the revenue process and understanding jurisdictional laws and regulations. With respect to estimates of amounts offset against sales and sales accruals, we obtained management's calculations and assessed the assumptions used by reference to the Group's stated commercial policies and the respective jurisdictional laws and regulations. Specifically, we considered the Group's processes for making judgments in this area and performed the following procedures:</p> <ul style="list-style-type: none"> <li>• analysed rebates and discounts accrued during the year against subsequent payments or releases;</li> <li>• analysed and recalculated components of the year-end liability based on contracted and statutory rebate and discount rates;</li> <li>• considered the experience from previous years, including evaluating changes in estimates for indicators of management bias, and assessing changes made to 31 December 2015 sales accruals based on new information that became available in 2016.</li> </ul>

**Other information in the annual report**

The Board of Directors is responsible for the other information in the annual report. The other information comprises all information included in the annual report, but does not include the consolidated financial statements, the stand-alone financial statements, the compensation report and our auditor's reports thereon.

Our opinion on the consolidated financial statements does not cover the other information in the annual report and we do not express any form of assurance conclusion thereon.

In connection with our audit of the consolidated financial statements, our responsibility is to read the other information in the annual report and, in doing so, consider whether the other information is materially inconsistent with the consolidated financial statements or our knowledge obtained in the audit, or otherwise appears to be materially misstated. If, based on the work we have performed, we conclude that there is a material misstatement of this other information, we are required to report that fact. We have nothing to report in this regard.

### **Responsibility of the Board of Directors for the consolidated financial statements**

The Board of Directors is responsible for the preparation of the consolidated financial statements that give a true and fair view in accordance with IFRS and the provisions of Swiss law, and for such internal control as the Board of Directors determines is necessary to enable the preparation of consolidated financial statements that are free from material misstatement, whether due to fraud or error.

In preparing the consolidated financial statements, the Board of Directors is responsible for assessing the Group's ability to continue as a going concern, disclosing, as applicable, matters related to going concern and using the going concern basis of accounting unless the Board of Directors either intends to liquidate the Group or to cease operations, or has no realistic alternative but to do so.

### **Auditor's responsibilities for the audit of the consolidated financial statements**

Our objectives are to obtain reasonable assurance about whether the consolidated financial statements as a whole are free from material misstatement, whether due to fraud or error, and to issue an auditor's report that includes our opinion. Reasonable assurance is a high level of assurance, but is not a guarantee that an audit conducted in accordance with Swiss law, ISAs and Swiss Auditing Standards will always detect a material misstatement when it exists. Misstatements can arise from fraud or error and are considered material if, individually or in the aggregate, they could reasonably be expected to influence the economic decisions of users taken on the basis of these consolidated financial statements.

A further description of our responsibilities for the audit of the consolidated financial statements is located at the website of EXPERTsuisse: <http://www.expertsuisse.ch/en/audit-report-for-public-companies>. This description forms part of our auditor's report.

### **Report on other legal and regulatory requirements**

In accordance with article 728a para. 1 item 3 CO and the Swiss Auditing Standard 890, we confirm that an internal control system exists, which has been designed for the preparation of consolidated financial statements according to the instructions of the Board of Directors.

We recommend that the consolidated financial statements submitted to you be approved.

Ernst & Young Ltd

/s/ Jolanda Dolente  
Licensed audit expert  
(Auditor in charge)

/s/ Frederik Schmachtenberg  
Licensed audit expert

# Statutory Financial Statements of Santhera Pharmaceuticals Holding AG

## Contents

Balance Sheet .....	63
Income Statement .....	64
Notes to the Statutory Financial Statements .....	65
1 Introduction .....	65
2 Principles .....	65
3 Information on Balance Sheet and Income Statement Items .....	66
4 Other Information .....	68
Proposal of the Board of Directors to the Annual Shareholders' Meeting .....	72
Report of the Statutory Auditor on the Financial Statements .....	73

## Balance Sheet

	As of December 31, in CHF thousands	Notes	2016	2015
<b>Assets</b>				
Cash and cash equivalents			43,187	61,256
Other receivables from third parties			24	43
Other receivables from shareholdings			0	287
Prepaid expenses and accrued income			46	180
<b>Current assets</b>			<b>43,257</b>	<b>61,766</b>
Loans to shareholdings		3.1	17,727	0
Investments in shareholding		3.2	115	59
<b>Noncurrent assets</b>			<b>17,842</b>	<b>59</b>
<b>Total assets</b>			<b>61,099</b>	<b>61,825</b>
<b>Liabilities and equity</b>				
Trade accounts payable to third parties			97	154
Other accounts payable to third parties			56	188
Other accounts payable to shareholdings			0	188
Accrued expenses			453	297
<b>Current liabilities</b>			<b>606</b>	<b>827</b>
<b>Total liabilities</b>			<b>606</b>	<b>827</b>
Share capital		3.3	6,280	6,263
<i>Reserves from capital contributions</i>			7,425	57,083
<i>Other capital reserves</i>			2,916	2,891
Statutory capital reserves			10,341	59,974
<i>Accumulated result</i>			-6,451	-5,557
<i>Results carried forward</i>			-5,557	-2,593
<i>Net result for the period</i>			-895	-2,964
<i>Other voluntary reserves (free reserves)</i>			50,495	495
Voluntary accumulated result and other reserves			44,044	-5,062
Treasury shares		3.4	-172	-177
<b>Total equity</b>			<b>60,493</b>	<b>60,998</b>
<b>Total liabilities and equity</b>			<b>61,099</b>	<b>61,825</b>

## Income Statement

For the year ended December 31, in CHF thousands	Notes	2016	2015
Income from shareholdings	3.5	1,551	1,970
Other operating income		134	1
<b>Total operating income</b>		<b>1,685</b>	<b>1,971</b>
General and administrative expenses	3.6	-2,159	-3,322
Employee costs		-792	-1,656
Other operating expenses		-25	-2
<b>Total operating expenses</b>		<b>-2,976</b>	<b>-4,980</b>
<b>Operating result</b>		<b>-1,291</b>	<b>-3,009</b>
Financial income		358	21
Financial expenses		-18	-35
<b>Financial result</b>		<b>340</b>	<b>-14</b>
Reversal on allowance of investment		56	59
<b>Result before taxes</b>		<b>-895</b>	<b>-2,964</b>
Direct taxes		0	0
<b>Net result</b>		<b>-895</b>	<b>-2,964</b>

# Notes to the Statutory Financial Statements

## 1 Introduction

Santhera Pharmaceuticals Holding AG (the Company or Santhera) is the parent company of Santhera Group. The Company has its registered offices at Hammerstrasse 49 in 4410 Liestal, Switzerland.

## 2 Principles

### General

The statutory financial statements of the Company are prepared in accordance with the general accepted accounting principles as set out in Art. 957 to Art. 963b, of the Swiss Code of Obligations (**CO**). Since Santhera prepares consolidated financial statements in accordance with International Financial Reporting Standards (**IFRS**) of the International Accounting Standards Board (**IASB**), a recognized accounting standard, the Company has, in accordance with the CO, elected to forego presenting the statement of cash flows, the additional disclosures and the management report otherwise required by the CO.

### Cash

Santhera holds cash balances, denominated mainly in Swiss francs (**CHF**) which include cash deposited in demand bank accounts, money market investment accounts and other liquid investments and interest earned on such cash balances.

### Current assets and liabilities

Current assets are recorded at historical cost less adjustments for impairment of value and current liabilities at historical cost.

### Loans to shareholdings

These are valued at their acquisition cost adjusted for impairment losses.

### Investments in shareholdings

Investments in shareholdings are recorded at acquisition cost less adjustments for impairment of value. We evaluate our investments in subsidiaries for impairment annually and record an impairment loss when the carrying amount of such assets exceeds the fair value. When estimating the fair value of our investments we base such valuation predominantly on the income approach.

## Treasury shares

Treasury shares are recognized at acquisition cost and deducted from shareholders' equity at the time of acquisition. Santhera holds treasury shares for market making which is maintained by an external bank. In case of a resale, the gain or loss is recognized through the income statement as financial income or financial expenses.

## Related parties

In the meaning of the New Swiss Accounting Law, we consider related parties to be only shareholders, direct and indirect subsidiaries (shareholdings) and the board of directors.

## 3 Information on Balance Sheet and Income Statement Items

### 3.1 Loans to shareholdings

Loans are granted to shareholdings primarily to fund the development and marketing activities of the Santhera Group (December 31, 2016: CHF 190.1 million; December 31, 2015: CHF 172.4 million). Until the end of 2015 the balance consisted of fully impaired and subordinated loans to Santhera Pharmaceuticals (Schweiz) AG. To finance the activities in development and the commercialization of LHON, in 2016 the loan granted to Santhera Pharmaceuticals were increased (with the additional loans also being subordinated). As part of the annual reassessment as of December 31, 2016, Executive Management concluded that approximately 10% of the total loan balance is recoverable considering a more positive outlook, both in terms of market success of the developed and launched product (Raxone in LHON) and the development progress in other indications (e.g. Raxone in DMD).

### 3.2 Investments in shareholdings

In 2016 and 2015, the following companies are direct subsidiaries of Santhera Pharmaceuticals Holding AG (100% ownership and 100% voting rights):

	Share capital at December 31	2016	2015
Santhera Pharmaceuticals (Schweiz) AG Liestal, Switzerland	CHF	125,000	125,000
Santhera Pharmaceuticals (Deutschland) GmbH Lörrach, Germany	EUR	25,000	25,000
Santhera Pharmaceuticals (USA), Inc. Burlington, US	USD	1,000	1,000
Santhera Pharmaceuticals (Canada), Inc. Montréal, Canada	CAD	1,000	1,000
Oy Santhera Pharmaceuticals (Finland) Ltd Helsinki, Finland	EUR	2,500	2,500

Santhera Pharmaceuticals (Schweiz) AG is the primary operational entity while Santhera Pharmaceuticals (Deutschland) GmbH holds the market authorization for the EU. Oy Santhera Pharmaceuticals (Finland) Ltd is not employing any personnel.

In 2015, the following companies, which are 100% direct subsidiaries (100% voting rights) of Santhera Pharmaceuticals (Schweiz) AG, were founded:

	Share capital at December 31	2016	2015
Santhera Pharmaceuticals (Liechtenstein) AG Ruggell, Fürstentum Liechtenstein	CHF	50,000	50,000
Santhera (Italy) S.r.l. Milano, Italy	EUR	50,000	50,000
Santhera (Germany) GmbH Munich, Germany	EUR	50,000	50,000
Santhera (Netherlands) B.V. Nieuwegein, The Netherlands	EUR	50,000	50,000
Santhera (UK) Limited London, United Kingdom	GBP	50,000	50,000

### 3.3 Share capital

During 2016, the share capital was increased by a total amount of CHF 17,059 to CHF 6,279,857 as of December 31, 2016 (2015: CHF 6,262,798) through the exercise of employee stock options (from conditional share capital).

### 3.4 Treasury shares

The movement of treasury shares held by Santhera was as follows:

	No of Shares	TCHF
January 1, 2015	8,028	177
December 31, 2015	8,028	177
Purchase	23,002	1,069
Sale <sup>1</sup>	-27,414	-1,074
December 31, 2016	3,616	172

<sup>1</sup> In connection with the liquidation of Oy Juvantia Pharma, Turku, Finland (**Juvantia**), acquired in 2009, Santhera received 8,028 shares from former Juvantia shareholders. These treasury shares served as pledge from the former owners of Juvantia for compensation of a potential tax claim related to pre-acquisition activities. The claim was resolved and the shares were sold with a financial profit of TCHF 186.

### 3.5 Income from shareholdings

Income from shareholdings represents reimbursement for management services provided by the Company to its major shareholding Santhera Pharmaceuticals (Schweiz) AG.

### 3.6 General and administrative expenses

	In CHF thousands	2016	2015
Administrative expenses		990	712
Consulting expenses		1,169	667
Expenses in connection with capital increases		0	1,943
<b>Total</b>		<b>2,159</b>	<b>3,322</b>

## 4 Other Information

### 4.1 Full-time equivalents

The number of full-time equivalents at period end was not above 10.

### 4.2 Significant shareholders (>2%)

Pursuant to information from the Company's share register and the disclosure of participations made to the Company in accordance with applicable stock exchange regulation, the following shareholders owned 2% or more of the Company's share capital as registered in the commercial register at December 31, 2016: 6,262,798 shares (February 11, 2016: 6,262,798 shares):

	2016 Shares <sup>1</sup>	2016 %	2015 Shares <sup>2</sup>	2015 %
Iglu Group, Switzerland <sup>3</sup>	632,300	10.1	671,858	10.7
Consonance Capital Management, US	597,069	9.5	625,457	10.0
Bertarelli Ernesto, Guichard-Bertarelli Donata and Bertarelli Maria-Iris, Switzerland <sup>3</sup>	545,777	8.7	545,777	8.7
UBS Fund Management (Switzerland) AG	195,007	3.1	n/a	n/a
Lagoda Investments Management, LLC, US	187,888	3.0	187,888	3.0
UBS Fund Management (Luxembourg) S.A.	183,699	2.9	167,203	2.7
Union Asset Management Holding AG	175,838	2.8	326,838	5.2
RTW Investments, LTD, US	146,365	2.3	140,354	2.2
Visium Balanced Master Fund, Ltd., US <sup>4</sup>	n/a	n/a	179,574	2.9

<sup>1</sup> Including disclosures until December 31, 2016

<sup>2</sup> Including disclosures until March 30, 2016

<sup>3</sup> On January 25, 2017, both Iglu Group and the Bertarelli Group announced that they had formed a new group with combined holdings of 1,179,977 Shares (18.8%).

<sup>4</sup> The fund was liquidated in June 2016 (Bloomberg, June 18, 2016).

### 4.3 Disclosure of shares and equity rights (share appreciation rights and stock options) held by members of the Board and Executive Management (and their respective related party)

*As of December 31, 2016:*

	Number of Shares	Number of vested equity rights	Number of unvested equity rights	Total number of equity rights
<i>Board of Directors</i>				
Martin Gertsch, Chairman	38,109	0	6,281	6,281
Jürg Ambühl	30,000	0	7,281	7,281
<i>Executive Management</i>				
Thomas Meier, CEO	72,902	3,750	14,875	18,625
Todd Bazemore, Chief Operating Officer US <sup>1</sup>	0	0	34,881	34,881
Nicholas Coppard, SVP Head Development	0	0	12,250	12,250
Günther Metz, SVP Business Development	0	12,000	7,120	19,120
Christoph Rentsch, Chief Financial Officer	0	0	22,000	22,000
Giovanni Stropoli, Chief Commercial Officer Europe and Rest of World	600	0	20,565	20,565
Oliver Strub, SVP General Counsel and Secretary to the Board	0	9,001	7,240	16,241

<sup>1</sup> Joined the Executive Management September 6, 2016.

*As of December 31, 2015:*

	Number of Shares	Number of vested equity rights	Number of unvested equity rights	Total number of equity rights
<i>Board of Directors</i>				
Martin Gertsch, Chairman	38,109	0	3,000	3,000
Jürg Ambühl	30,000	0	4,000	4,000
<i>Executive Management</i>				
Thomas Meier, CEO	72,902	0	12,250	12,250
Nicholas Coppard, SVP Head Development <sup>1</sup>	1	0	9,000	9,000
Günther Metz, SVP Business Development <sup>1</sup>	0	11,000	5,000	16,000
Christoph Rentsch, Chief Financial Officer <sup>2</sup>	0	0	15,000	15,000
Giovanni Stropoli, Chief Commercial Officer Europe and Rest of World <sup>1</sup>	400	0	15,000	15,000
Oliver Strub, SVP General Counsel and Secretary to the Board <sup>1</sup>	0	10,000	5,000	15,000

<sup>1</sup> Joined the Executive Management February 1, 2015.

<sup>2</sup> Joined the Executive Management July 1, 2015.

#### 4.4. Disclosure of the allocation of equity rights for Board of Directors, Executive Management and employees of Santhera Group

	2016	2016	2015	2015
	Quantity	Value (in TCHF) <sup>1</sup>	Quantity	Value (in TCHF) <sup>1</sup>
Board of Directors	6,562	224	7,000	282
Executive Management	65,431	1,349	53,500	2,094
Employees of Santhera Group	126,980	3,121	88,760	3,612
<b>Total</b>	<b>198,973</b>	<b>4,694</b>	<b>149,260</b>	<b>5,988</b>

<sup>1</sup> Value of the equity rights calculated in accordance with the Hull-White model at the date of allocation in accordance with the terms of the award. The tax value of equity rights is 0 until they would be exercised. Such equity rights values are theoretical values and do not reflect income tax values and do also take into consideration certain vesting provisions. For information about the underlying equity rights plans, see note 17 "Equity Rights Plans" in the consolidated financial statements. For information about the Company's compensation procedures, consult the Corporate Governance Report and the Compensation Report.

On January 1, 2017, 198,162 share appreciation rights (SARs) are planned to be conditionally granted to employees of Santhera. These SARs are part of the bonus award for the year 2016 to employees of the Group. These SARs were granted under ESARP 2016 (see note 17 "Equity Rights Plans").

	Quantity	Value (in TCHF) <sup>1</sup>
Executive Management	59,860	1,330
Employees of Santhera Group	138,302	3,072
<b>Total</b>	<b>198,162</b>	<b>4,402</b>

<sup>1</sup> Value of the equity rights calculated in accordance with the Hull-White model at the date of allocation in accordance with the terms of the award. The tax value of equity rights is 0 until they would be exercised. Such equity rights values are theoretical values and do not reflect income tax values and do also take into consideration certain vesting provisions. For information about the underlying equity rights plans, see note 17 "Equity Rights Plans" in the consolidated financial statements. For information about the Company's compensation procedures, consult the Corporate Governance Report and the Compensation Report.

#### 4.5 Contingencies and guarantees

##### *Guarantee towards Swiss VAT authorities*

The Company is part of the value-added tax group of the Swiss affiliated companies of Santhera Pharmaceuticals and is therefore jointly and severally liable to the Swiss federal tax administration for their value-added tax liabilities.

##### *Guarantee towards Santhera Pharmaceuticals (Schweiz) AG*

The Company guarantees to pay for the liabilities of its subsidiary Santhera Pharmaceuticals (Schweiz) AG until the Annual Shareholders' Meeting in 2018.

##### *Declaration of liability towards Arval Deutschland GmbH*

The Company guarantees to pay for the liabilities of its subsidiary Santhera (Germany) GmbH for contractual duties and obligations.

#### 4.6 Events after the reporting date

On January 25, 2017, Santhera announced that it has received from Ernesto Bertarelli, Donata Guichard-Bertarelli, Maria-Iris Bertarelli, (together, the "Bertarelli Group") and Ralf Arnold, Markus Kühnle and Thomas Terhorst (together, the "Iglu Group") the notification that they have combined their respective share-holdings in Santhera to form a new shareholder group. The above mentioned shareholders have announced that they have formed a new shareholder group with a combined holding in Santhera of 18.84% (1,179,977 shares).

On February 10, 2017, Santhera has successfully placed CHF 60 million senior unsecured convertible bonds (the "Convertible Bonds") due 2022. The Company intends to use the net proceeds from this placement primarily to fund the commercialization of Raxone in the currently approved indication, to prepare the market entry and commercial launch in subsequent indications, for investment into further clinical trials with Raxone and for other corporate purposes.

The Convertible Bonds have a 5-year maturity and a coupon of 5.00% per annum. The conversion price was fixed at CHF 86.4006, representing a premium of 20% over the volume weighted average price (**VWAP**) of the Shares between the launch and pricing of the Convertible Bonds (the "Reference Share Price"). The Convertible Bonds are issued at 100% of their principal amount and, unless previously redeemed, converted or repurchased and cancelled, will mature on February 17, 2022, at 100% of their principal amount.

The Conversion Price will be reset after the first year if the VWAP of the shares during a specified period of time will be below the Reference Share Price. The new Conversion Price must not be lower than 75% of the Conversion Price at issuance. In addition, Santhera may call the Convertible Bonds at any time on or after the second anniversary of the issue date at par, plus accrued interest, if any, if the VWAP of the shares is at least 160% of the Conversion Price.

The Convertible Bonds will be convertible into 694,440 registered Shares of Santhera Pharmaceuticals Holding AG, representing 11.1% of the current outstanding share capital of Santhera Pharmaceuticals Holding AG. The Shares to be delivered upon conversion shall be sourced from conditional and, if needed, from authorized capital.

The Convertible Bonds are listed and traded on the SIX Swiss Exchange since February 16, 2017.

Santhera agreed to a company lock-up ending 90 days after that date, subject to customary exceptions.

## Report of the Statutory Auditor on the Financial Statements

Basle, 6 March 2017

As statutory auditor, we have audited the financial statements of Santhera Pharmaceuticals Holding Ltd, which comprise the balance sheet, income statement and notes (pages 63 to 71), for the year ended 31 December 2016.

### **Board of Directors' responsibility**

The Board of Directors is responsible for the preparation of the financial statements in accordance with the requirements of Swiss law and the company's articles of incorporation. This responsibility includes designing, implementing and maintaining an internal control system relevant to the preparation of financial statements that are free from material misstatement, whether due to fraud or error. The Board of Directors is further responsible for selecting and applying appropriate accounting policies and making accounting estimates that are reasonable in the circumstances.

### **Auditor's responsibility**

Our responsibility is to express an opinion on these financial statements based on our audit. We conducted our audit in accordance with Swiss law and Swiss Auditing Standards. Those standards require that we plan and perform the audit to obtain reasonable assurance whether the financial statements are free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the financial statements. The procedures selected depend on the auditor's judgment, including the assessment of the risks of material misstatement of the financial statements, whether due to fraud or error. In making those risk assessments, the auditor considers the internal control system relevant to the entity's preparation of the financial statements in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the entity's internal control system. An audit also includes evaluating the appropriateness of the accounting policies used and the reasonableness of accounting estimates made, as well as evaluating the overall presentation of the financial statements. We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our audit opinion.

### **Opinion**

In our opinion, the financial statements for the year ended 31 December 2016 comply with Swiss law and the company's articles of incorporation.

### **Report on key audit matters based on the circular 1/2015 of the Federal Audit Oversight Authority**

Key audit matters are those matters that, in our professional judgement, were of most significance in our audit of the financial statements of the current period. These matters were addressed in the context of our audit of the financial statements as a whole, and in forming our opinion thereon, and we do not provide a separate opinion on these matters. For each matter below, our description of how our audit addressed the matter is provided in that context.

We have fulfilled the responsibilities described in the *Auditor's responsibilities* section of our report, including in relation to these matters. Accordingly, our audit included the performance of procedures designed to respond to our assessment of the risks of material misstatement of the financial statements. The results of our audit procedures, including the procedures performed to address the matters below, provide the basis for our audit opinion on the financial statements.

### Valuation of investments in and long-term receivables from shareholdings

<b>Area of focus</b>	Santhera Pharmaceuticals Holding Ltd holds investments in subsidiaries and grants loans to subsidiaries for financing purposes, both of which are assessed for impairment as of the balance sheet date. We focused on this area because the impairment assessment of these investments and loans requires estimation and judgement around assumptions used, including prospective financial information and discount rates. Changes to assumptions could lead to material changes in the estimated recoverable amount, impacting both potential impairment charges and also potential reversals of impairment.
----------------------	--

*See note 3.1 and 3.2 to these financial statements for Santhera Pharmaceuticals Holding Ltd disclosures related to investment in and long-term receivables from shareholdings.*

<b>Our audit response</b>	We evaluated management's impairment indicator assessment, which is based on the income approach, under the applicable accounting standards and analysed the underlying key assumptions in connection with the prospective financial information and discount rates. We assessed the historical accuracy of the estimates and considered the ability to produce accurate long-term forecasts. We evaluated the sensitivity in the impairment indicator assessment resulting from changes to the key assumptions applied and compared these assumptions to externally available market information.
---------------------------	--

### Report on other legal requirements

We confirm that we meet the legal requirements on licensing according to the Auditor Oversight Act (AOA) and independence (article 728 CO and article 11 AOA) and that there are no circumstances incompatible with our independence.

In accordance with article 728a para. 1 item 3 CO and Swiss Auditing Standard 890, we confirm that an internal control system exists, which has been designed for the preparation of financial statements according to the instructions of the Board of Directors.

We recommend that the financial statements submitted to you be approved.

Ernst & Young Ltd

/s/ Jolanda Dolente  
Licensed audit expert  
(Auditor in charge)

/s/ Frederik Schmachtenberg  
Licensed audit expert

# Consolidated Financial Statements

## Contents

Consolidated Balance Sheet .....	12
Consolidated Income Statement .....	13
Consolidated Statement of Comprehensive Income .....	14
Consolidated Cash Flow Statement .....	15
Consolidated Statement of Changes in Equity .....	16
Notes to the Consolidated Financial Statements .....	17
1 General Information .....	17
2 Summary of Significant Accounting Policies .....	17
3 Critical Accounting Estimates, Assumptions and Judgments .....	26
4 Exchange Rates of Principal Currencies .....	26
5 Tangible Assets .....	27
6 Intangible Assets .....	28
7 Impairment Test for Intangible Assets .....	29
8 Prepaid Expenses and Accrued Income .....	30
9 Inventories .....	30
10 Trade and Other Receivables .....	30
11 Cash and Cash Equivalents .....	31
12 Share Capital .....	31
13 Deferred Taxes .....	33
14 Trade and Other Payables .....	34
15 Accrued Expenses .....	34
16 Commitments and Contingent Liabilities .....	34
17 Stock Option Plans .....	36
18 Segment and Geographic Information .....	39
19 Other Operating Income .....	40
20 Operating Expenses by Nature .....	40

21	Employee Expenses and Benefits .....	41
22	Financial Income/Expenses .....	44
23	Income Taxes .....	44
24	Earnings/Loss per Share .....	45
25	Related Party Transactions .....	45
26	Risk Management Objectives and Policies .....	46
27	Events After the Reporting Date .....	49
	Report of the Statutory Auditor on the Consolidated Financial Statements .....	50

## Consolidated Balance Sheet

	In CHF thousands	Notes	31.12.2015	31.12.2014 Restated <sup>1</sup>
<b>Assets</b>				
Tangible assets		5	398	132
Intangible assets		6	29,559	4,274
Financial assets long-term			190	85
Deferred tax assets		13	3,061	0
<b>Noncurrent assets</b>			<b>33,208</b>	<b>4,491</b>
Prepaid expenses and accrued income		8	1,513	376
Inventories		9	3,441	0
Trade and other receivables		10	2,131	720
Cash and cash equivalents		11	76,859	17,435
<b>Current assets</b>			<b>83,944</b>	<b>18,531</b>
<b>Total assets</b>			<b>117,152</b>	<b>23,022</b>
<b>Equity and liabilities</b>				
Share capital		12	6,263	4,974
Capital reserves and share premium			377,031	293,650
Retained earnings			-273,133	-279,083
Employee benefit reserve			-2,958	-1,287
Treasury shares		12	-177	-177
Other components of equity			-779	-762
<b>Total equity</b>			<b>106,247</b>	<b>17,315</b>
Pension liabilities		21	3,957	2,680
<b>Total noncurrent liabilities</b>			<b>3,957</b>	<b>2,680</b>
Trade and other payables		14	3,666	2,166
Accrued expenses		15	3,282	861
<b>Total current liabilities</b>			<b>6,948</b>	<b>3,027</b>
<b>Total liabilities</b>			<b>10,905</b>	<b>5,707</b>
<b>Total equity and liabilities</b>			<b>117,152</b>	<b>23,022</b>

<sup>1</sup> Some positions have been restated, see note 2 "Correction of errors".

## Consolidated Income Statement

For the year ended December 31, in CHF thousands	Notes	2015	2014 Restated <sup>1</sup>
Net sales	18	4,321	2,591
Cost of goods sold		-1,371	-199
Other operating income	19	188	533
Development	20	16,651	-5,876
<i>Of which Development expenses</i>	20	-10,453	-5,876
<i>Of which reversal impairment on intangible assets and inventory</i>	20	27,104	0
Marketing and sales	20	-8,356	-580
General and administrative	20	-8,244	-4,395
Other operating expenses	20	-16	-9
<b>Operating expenses</b>	<b>20</b>	<b>35</b>	<b>-10,860</b>
<b>Operating result</b>		<b>3,173</b>	<b>-7,935</b>
Financial income	22	416	54
Financial expenses	22	-655	-69
<b>Result before taxes</b>		<b>2,934</b>	<b>-7,950</b>
Income taxes	23	3,015	-2
<b>Net result</b>		<b>5,949</b>	<b>-7,952</b>
Basic earnings/loss per share (in CHF)	24	1.11	-1.69
Diluted earnings/loss per share (in CHF)	24	1.08	-1.69

<sup>1</sup> Some positions have been restated, see note 2 "Correction of errors".

## Consolidated Statement of Comprehensive Income

For the year ended December 31, in CHF thousands	Notes	2015	2014 Restated <sup>1</sup>
<b>Net result</b>		<b>5,949</b>	<b>-7,952</b>
<i>Items never to be reclassified to net income in subsequent periods:</i>			
Actuarial gains/(losses) on defined benefit plans	21	-1,671	-1,692
<i>Items to be reclassified to net income in subsequent periods:</i>			
Currency translation differences		-16	5
<b>Other comprehensive result</b>		<b>-1,687</b>	<b>-1,687</b>
<b>Total comprehensive result</b>		<b>4,262</b>	<b>-9,639</b>

<sup>1</sup> Some positions have been restated, see note 2 "Correction of errors".

## Consolidated Cash Flow Statement

For the year ended December 31, in CHF thousands	Notes	2015	2014 Restated <sup>1</sup>
Result before taxes		<b>2,934</b>	<b>-7,950</b>
Depreciation of tangible assets	5	85	66
Reversal of impairment on intangible assets	2, 6	-26,157	0
Amortization of intangible assets	6	1,037	9
Expenses for share options	17, 20	2,040	1,177
Change in pension liabilities	21	-394	-9
Change in deferred taxes	13	-3,061	0
Taxes paid		-46	-2
Change in net working capital		942	634
Total financial result	22	239	15
Interest received	22	2	4
Interest paid	22	-11	-7
<b>Cash flow from operating activities</b>		<b>-22,390</b>	<b>-6,063</b>
Investments in tangible assets	5	-350	-160
Investments in intangible assets	6	-165	-47
Investments in other financial assets		-104	0
<b>Cash flow from investing activities</b>		<b>-619</b>	<b>-207</b>
Capital increases from options exercised	12	2,127	3,247
Proceeds from sale of treasury shares SEDA <sup>2</sup>	12	0	1,444
Capital increase private placement	12	54,870	1,000
Capital increase	12	27,576	13,294
Cost of issuance of share capital		-1,943	-324
<b>Cash flow from financing activities</b>		<b>82,630</b>	<b>18,661</b>
Effects of exchange rate changes on cash and cash equivalents		-197	0
<b>Net increase/(decrease) in cash and cash equivalents</b>		<b>59,424</b>	<b>12,391</b>
Cash and cash equivalents at January 1		17,435	5,044
<b>Cash and cash equivalents at December 31</b>		<b>76,859</b>	<b>17,435</b>

<sup>1</sup> Some positions have been restated, see note 2 "Correction of errors".

<sup>2</sup> Standby Equity Distribution Agreement, see note 12 "Share Capital".

## Consolidated Statement of Changes in Equity

In CHF thousands	Notes	Share capital	Capital reserves and share premium	Retained earnings	Employee benefit reserve	Treasury shares	Translation differences	Total
<b>Balance at January 1, 2014 (as previously reported)</b>		<b>3,934</b>	<b>274,896</b>	<b>-265,304</b>	<b>405</b>	<b>-221</b>	<b>-6,604</b>	<b>7,106</b>
Correction	2	0	0	-5,827	0	0	5,837	10
<b>Balance at January 1, 2014 (after correction<sup>1</sup>)</b>		<b>3,934</b>	<b>274,896</b>	<b>-271,131</b>	<b>405</b>	<b>-221</b>	<b>-767</b>	<b>7,116</b>
Net result <sup>1</sup>		0	0	-7,952	0	0	0	-7,952
Other comprehensive result		0	0	0	-1,692	0	5	-1,687
<b>Total comprehensive result for the period</b>		<b>0</b>	<b>0</b>	<b>-7,952</b>	<b>-1,692</b>	<b>0</b>	<b>5</b>	<b>-9,639</b>
Share-based payment transactions <sup>1</sup>		0	1,177	0	0	0	0	1,177
Capital increase from options exercise		197	3,050	0	0	0	0	3,247
Capital increase SEDA <sup>2</sup>		355	1,045	0	0	44	0	1,444
Capital increase private placement		288	712	0	0	0	0	1,000
Capital increase		200	13,094	0	0	0	0	13,294
Cost of issuance of share capital		0	-324	0	0	0	0	-324
<b>Balance at December 31, 2014<sup>1</sup></b>		<b>4,974</b>	<b>293,650</b>	<b>-279,083</b>	<b>-1,287</b>	<b>-177</b>	<b>-762</b>	<b>17,315</b>
<b>Balance at January 1, 2015</b>		<b>4,974</b>	<b>293,650</b>	<b>-279,083</b>	<b>-1,287</b>	<b>-177</b>	<b>-762</b>	<b>17,315</b>
Net result		0	0	5,949	0	0	0	5,949
Other comprehensive result		0	0	0	-1,671	0	-16	-1,687
<b>Total comprehensive result for the period</b>		<b>0</b>	<b>0</b>	<b>5,949</b>	<b>-1,671</b>	<b>0</b>	<b>-16</b>	<b>4,262</b>
Share-based payment transactions		0	2,040	0	0	0	0	2,040
Capital increase from options exercise		399	1,728	0	0	0	0	2,127
Capital increase private placement		590	54,280	0	0	0	0	54,870
Capital increase		300	27,276	0	0	0	0	27,576
Cost of issuance of share capital		0	-1,943	0	0	0	0	-1,943
<b>Balance at December 31, 2015</b>		<b>6,263</b>	<b>377,031</b>	<b>-273,134</b>	<b>-2,958</b>	<b>-177</b>	<b>-778</b>	<b>106,247</b>

<sup>1</sup> Some positions have been restated, see note 2 "Correction of errors".

<sup>2</sup> Standby Equity Distribution Agreement, see note 12 "Share Capital".

# Notes to the Consolidated Financial Statements

## 1 General Information

Santhera Pharmaceuticals Holding AG (the **Company**, together with its subsidiaries **Santhera** or **Group**) is a specialty pharmaceutical company focused on the development and commercialization of products for the treatment of mitochondrial and neuromuscular diseases, an area which includes many orphan and niche indications with no current therapy.

The Company, having its primary listing of its registered shares (**Shares**) on the SIX Swiss Exchange (**SIX**), is a Swiss stock corporation and the parent company of the Group. Its purpose is to acquire, dispose and manage investments. The Company has its registered offices at Hammerstrasse 49 in 4410 Liestal, Switzerland.

The consolidated financial statements were approved for publication by the Board of Directors (**Board**) on April 11, 2016. They are subject to approval by the Annual Shareholders' Meeting (**ASM**) on May 11, 2016.

## 2 Summary of Significant Accounting Policies

The principal accounting policies applied in the preparation of these financial statements are set out below. These policies have been consistently applied to all the years presented, unless otherwise stated.

### Basis of preparation

The consolidated financial statements of Santhera have been prepared in accordance with International Financial Reporting Standards (**IFRS**).

The consolidated financial statements are based on the financial statements of the individual Santhera companies prepared for the same reporting period using consistent accounting policies. The consolidated financial statements are prepared using the historical cost convention except for the revaluation to fair value of certain financial assets and financial liabilities.

The presentation currency is Swiss francs (**CHF**). All figures included in these financial statements and notes to the financial statements are rounded to the nearest CHF 1,000 except where otherwise indicated.

## Consolidation

Subsidiaries in which the Company has a direct or indirect controlling interest are consolidated. Control exists when the investor is exposed, or has rights, to variable returns from its investment with the investee and has the ability to affect those returns through its power over the investee. Control is normally evidenced when the Company owns, either directly or indirectly, more than 50% of the voting rights or potential voting rights of a company's share capital that are currently exercisable.

The consolidated financial statements of Santhera include the accounts of Santhera Pharmaceuticals Holding AG, Liestal, Switzerland, and its wholly owned subsidiaries Santhera Pharmaceuticals (Schweiz) AG, Liestal, Switzerland; Santhera Pharmaceuticals (USA), Inc., Charlestown, US; Santhera Pharmaceuticals (Canada), Inc., Montréal, Canada; Santhera Pharmaceuticals (Deutschland) GmbH, Lörrach, Germany; and Oy Santhera Pharmaceuticals (Finland) Ltd, Helsinki, Finland. The accounts further include the wholly owned subsidiaries of Santhera Pharmaceuticals (Schweiz) AG: Santhera Pharmaceuticals (Liechtenstein) AG, Ruggell, Fürstentum Liechtenstein; Santhera (Italy) S.r.l., Milano, Italy; Santhera (Germany) GmbH, Munich, Germany; Santhera (Netherlands) B.V., Nieuwegein, The Netherlands; and Santhera (UK) Limited, London, United Kingdom.

Consolidation commences from the date on which control is transferred to the Company, and subsidiaries are no longer consolidated from the date that control ceases. Intercompany balances and transactions between Group companies are eliminated. Intercompany transactions solely result from providing services, financing and selling goods to other Group companies.

## Correction of errors

In the context of the annual impairment testing for its intangible assets, Santhera became aware that the previously impaired intangible asset "Raxone/Catena" had been reported in EUR rather than CHF, generating exchange differences in other comprehensive income that had accumulated in other components of equity over the years to CHF 5.8 million.

The comparative figures for the year 2014 were corrected retrospectively in accordance with IAS 8. The correction of the error did not have an impact on the consolidated income statement and consolidated cash flow statement. The overall net impact on the consolidated balance sheet was considered not material and no third balance sheet as at January 1, 2014, has been presented.

In the context of the preparation of the compensation report 2015, Santhera became aware that part of the expenses for employee stock options granted in 2015 should have been accounted for in the year 2014. Although these options were only granted in 2015, they formed part of the bonus award to employees for the year ended December 31, 2014, and employees had been rendering services in 2014 in expectation of the annual bonus allocation. Executive management determined that the award should have been expensed starting from 2014 until the date of vesting.

The comparative figures for the year 2014 were corrected retrospectively in accordance with IAS 8. The correction of this error did not have a net impact on the consolidated balance sheet, consolidated cash flow statement and consolidated statement of changes in equity. The overall impact on the consolidated income statement of 2014 is an increase of operating expenses of TCHF 418.

The impact on the relevant positions of the Group's prior year consolidated balance sheet, income statement and consolidated statement of comprehensive income is shown below:

### Consolidated balance sheet

In CHF thousands	January 1, 2014 reported	Intangible assets	Employee stock options	January 1, 2014 restated
Assets:				
Intangible assets	4,225	10	-	4,235
Equity:				
Retained earnings	-265,304	-5,827	-	-271,131
Other components of equity	-6,604	5,837	-	-767
Total equity	7,106	10	-	7,116

### Consolidated income statement and consolidated statement of comprehensive income

	2014 reported	Intangible assets	Employee stock options	2014 restated
Development	-5,695	-	-181	-5,876
Marketing and sales	-574	-	-6	-580
General and administrative	-4,164	-	-231	-4,395
Operating expenses	-10,442	-	-418	-10,860
Operating result	-7,517	-	-418	-7,935
Result before taxes	-7,532	-	-418	-7,950
Net result	-7,534	-	-418	-7,952
Currency translation differences	-62	67	-	5
Total comprehensive result	-9,288	67	-418	-9,639

### Consolidated balance sheet

In CHF thousands	December 31, 2014 reported	Intangible assets	Employee stock options	December 31, 2014 restated
Assets:				
Intangible assets	4,197	77	-	4,274
Equity:				
Capital reserves and share premium	293,232	-	418	293,650
Retained earnings	-272,838	-5,827	-418	-279,083
Other components of equity	-6,666	5,904	-	-762
Total equity	17,238	77	-	17,315

## Changes in accounting policies

The adopted accounting policies are consistent with the previous year except for those described below.

The following changes in standards had neither an effect on accounting policies nor on reported amounts or disclosures in these financial statements:

- IAS 19 (Amendments) Defined Benefit Plans: Employee Contributions (effective July 1, 2014)
- Annual Improvements (2011–2013 Cycle/2010–2012 Cycle) (effective July 1, 2014)

The IASB has issued a number of amendments to existing standards as well as new standards and interpretations which will become effective in future periods. Many of these changes are not relevant for Santhera or are currently not expected to have a material impact on Santhera's accounting policies or financial performance but may lead to additional disclosures. The most important change relates to IFRS 15 Revenue from Contracts with Customers and IFRS 16 Leases.

The Group is currently evaluating the impact of these changes on the Group's financial reporting:

- IFRS 9 (2014) Financial Instruments (effective January 1, 2018)
- IFRS 15 Revenue from Contracts with Customers (effective January 1, 2018)
- IFRS 16 Leases (effective January 1, 2019)
- IAS 1 (Amendments) Disclosure Initiative (effective January 1, 2016)
- IAS 7 (Amendments) Disclosure Initiative (effective January 1, 2017)
- IAS 16 and IAS 38 (Amendments) Clarification of Acceptable Methods of Depreciation and Amortization (effective January 1, 2016)
- Annual Improvements (2012–2014 Cycle) (effective January 1, 2016)

## Segment reporting

Santhera has one operating segment, namely the development and commercialization of products for the treatment of mitochondrial and neuromuscular diseases. The Board, the Executive Management and senior managers, being the Chief Operating Decision Makers (**CODM**), assess the reporting data and allocate resources as one segment on an aggregated consolidated level according to operating expenses by function. Santhera generates revenue from sales of Raxone<sup>®</sup> and Catena<sup>®</sup> (for the treatment of LHON and DMD). Geographic revenue information is based on location of the customer or licensee.

## Foreign currency translations

The consolidated financial statements are presented in CHF. The functional currency of each of Santhera's companies is the currency of the primary economic environment in which the local entity operates. Transactions in foreign currencies are accounted for at the rates prevailing at the dates of the transaction. Translation differences from financial transactions are included in the financial result.

Gains and losses resulting from the translation of foreign currency transactions and from the adjustment of foreign currency monetary assets and liabilities at the reporting date are recognized in the income statement.

Assets and liabilities of foreign entities are translated into CHF using the balance-sheet exchange rates at year-end. Income and expenses are translated into CHF at average exchange rates. The exchange differences arising on the retranslation are accounted for in other comprehensive income/equity.

### **Intangible assets**

Patents, licenses, trademarks and other intangible assets are capitalized as intangible assets when it is probable that future economic benefits will be generated. Such assets are in general amortized on a straight-line basis over their useful lives. Estimated useful life is the lower of legal duration and economic useful life. The estimated useful life of the intangible assets is regularly reviewed and if necessary the future amortization charge is accelerated. For pharmaceutical products, the estimated useful life normally corresponds to the remaining lifetime of their patent or orphan drug protection (up to 20 years).

### **Patents**

Patents not yet available for use are not amortized, but tested for impairment annually. Once useful life can be determined, amortization starts on a straight-line basis (2 to 20 years).

### **IT software**

Acquired IT software licenses are capitalized on the basis of the costs incurred to acquire and implement the specific software. These costs are amortized on a straight-line basis over their estimated useful lives (2 to 5 years).

### **Tangible assets**

Tangible assets are stated at cost less accumulated depreciation and any impairment losses. Depreciation is calculated on a straight-line basis over the estimated useful life of the asset or the shorter lease term, as follows:

	Useful life
Equipment	4 to 10 years
IT hardware	2 to 5 years

### **Impairment of assets**

Assets include intangible assets not yet available for use, intangible assets with finite useful lives and tangible assets. In general and in accordance with the terms of IFRS, assets not in use are capitalized at cost in the balance sheet and reviewed for impairment at least annually. Impairment testing is performed at the same time every year or whenever there is an indication that the asset may be impaired. A change to finite useful life is accounted for as a change in an accounting estimate for the respective asset. Testing for indicators of impairment is done at the end of each reporting period.

### **Trade and other receivables**

Receivables which generally have 30 days payment terms are stated at their nominal value less an allowance for any uncollectible amount if required. An allowance for doubtful debts is made when collection of the full amount is no longer probable.

### **Inventories**

Inventories are stated at the lower of cost and net realizable value using the weighted average cost formula.

### **Financial assets**

Generally, Santhera classifies its financial assets in the following categories:

#### *Financial assets at fair value through profit or loss*

This category has two subcategories: financial assets held for trading and those designated at fair value through profit or loss upon initial recognition. A financial asset is classified in this category if acquired principally for the purpose of selling in the short term. Assets in this category are classified as current assets if they are either held for trading or are expected to be realized within 12 months of the reporting date. Valuation is at fair value through profit and loss. Financial assets at fair value through profit or loss are subsequently carried at fair value. Realized and unrealized gains and losses arising from changes in the fair value are included in the income statement in the period in which they arise.

#### *Loans and receivables*

Loans and receivables are nonderivative financial assets with fixed or determinable payments that are not quoted in an active market. They arise when Santhera provides money, goods or services directly to a debtor with no intention of trading the receivable. They are included in current assets, except for maturities longer than 12 months after the balance sheet date. These are classified as non-current assets. Loans and receivables are measured at amortized cost using the effective interest method.

### **Leases**

Leases of assets under which Santhera essentially assumes all the rewards and risks of ownership are classified as finance leases. Finance leases are capitalized as assets and liabilities at the commencement of the lease at the fair value of the leased item or, if lower, at the present value of the minimum lease payments. The assets acquired under these contracts are depreciated over the shorter of the estimated useful life of the asset or the lease term.

Leases of assets under which the risks and rewards of ownership are effectively retained by the lessor are classified as operating leases, and payments made are charged to the income statement on a straight-line basis.

### **Cash and cash equivalents**

This item includes cash on hand and at banks, deposits held at call with banks and other short-term highly liquid investments with original maturities of three months or less.

## Share capital

Common shares are classified as equity. Incremental costs directly attributable to the issue of new common shares or options are shown in equity in the capital reserves and share premium as a deduction, net of tax, from the proceeds.

## Financial liabilities

Santhera classifies its financial liabilities into two categories:

### *Financial liabilities at fair value through profit or loss*

This category includes derivatives with negative replacement values. They are initially recognized at their fair value. Any subsequent change in fair value is recognized in the income statement in the period they occur.

### *Other liabilities measured at amortized costs*

This category principally covers debt instruments and trade and other payables. They are initially recognized at fair value and subsequently measured at amortized costs using the effective interest method. Any difference between the net proceeds received and the principal value due on redemption is amortized over the duration of the debt instrument and is recognized as part of interest expense in the income statement.

## Income taxes

The income tax charge is based on profit for the year and includes deferred taxes. Deferred taxes are calculated using the liability method. Deferred income taxes reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Deferred tax assets and liabilities are measured using the tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled based on enacted or substantially enacted tax rates as of the balance sheet date.

The amount of deferred tax liabilities and deferred tax assets reflects the tax consequences on the balance sheet date of the Company's expectation of recovery or settlement of such carrying amount of its assets and liabilities.

Deferred tax assets and liabilities are not discounted and are classified as noncurrent assets (liabilities) in the balance sheet. They are offset against each other if they relate to the same taxable entity and tax authority.

Deferred tax assets are recognized when it is probable that sufficient taxable profits will be available against which the deferred tax assets can be utilized. At each balance sheet date, the Company reassesses unrecognized deferred tax assets and the carrying amount of deferred tax assets. The Company recognizes a previously unrecognized deferred tax asset to the extent that it has become probable that future taxable profit will allow the deferred tax asset to be recovered. The Company conversely reduces the carrying amount of a deferred tax asset to the extent that it is no longer probable that sufficient taxable profit will be available to allow the benefit of part or the entire deferred tax asset to be utilized. Deferred tax is provided on temporary differences arising on investments in subsidiaries, associates and joint ventures, except where the timing of the reversal of the temporary difference can be controlled and it is probable that the difference will not reverse in the foreseeable future.

## Earnings/loss per share

Basic earnings/loss per share are calculated by dividing the net profit/loss attributable to owners of ordinary Shares of the Company by the weighted average number of Shares outstanding during the reporting period. Diluted earnings per share are calculated by dividing the net profit attributable to owners of ordinary Shares of the Company by the weighted average number of shares issued and outstanding during the reporting period adjusted for Shares held as treasury shares (purchased at market) and the number of potential shares from stock option plans.

## Employee benefits

### *Post-retirement benefits*

Santhera operates both defined benefit and defined contribution pension schemes.

- Defined benefit scheme:

Santhera's pension plan in Switzerland is classified as a defined benefit plan. Payments under this scheme are made directly to the pension fund for the account of each insured person. Typically, on retirement, an employee will receive an amount of the accumulated defined benefit obligation depending on several factors such as the total individual amount paid in, age and implied life expectancy. The compensation will be in the form of a lifelong pension or a lump sum payment. The scheme also covers disability as a consequence of illness and death-in-service.

The liability recognized in the balance sheet in respect of defined benefit pension plans is the present value of the defined benefit obligation at the balance sheet date less the fair value of plan assets, adjusted for the effects of the asset ceiling, when relevant.

The defined benefit obligation is calculated annually by independent actuaries using the projected unit credit method. The present value of the defined benefit obligation is determined by discounting the estimated future cash outflows using interest rates of high-quality corporate bonds that are denominated in the currency in which the benefits will be paid and that have terms to maturity approximating the terms of the related pension liability.

- Defined contribution schemes:

Defined contribution schemes are also funded through direct payments for the account of each insured person. Upon retirement, an employee will receive an amount of the accumulated contributions in the form of a lifelong pension or a lump sum payment. No further obligations arise from these schemes other than the fixed periodic contributions to the plan.

### *Share-based compensation*

Santhera has established several stock option plans to align the long-term interests of the members of the Board, the Executive Management, employees and selected consultants who are eligible to participate. Options granted under all plans are equity-settled. The fair value of employee stock options is determined at the grant date and recognized as personnel expense over the period Santhera receives services for each award. Where stock option awards are modified as a minimum, the expenses are recognized as if no terms had been modified; modifications which increase the fair value of options are expensed additionally. Unless determined otherwise by the Board, terminations of employment by the employer are treated as forfeiture and any previously accumulated share-based payment expenses for unvested awards are reversed.

## **Provisions**

Provisions are recognized when Santhera has a present obligation (legal or constructive) as a result of a past event, where it is more probable than not that a cash outflow will be required to fulfill the obligation and where a reliable estimate can be made of the amount of the obligation.

If the effect of the time value of money is material, provisions are determined by discounting the expected future outflows.

## **Revenue recognition**

Revenue comprises the fair value of the sale of goods and services, net of value-added tax, rebates, discounts, returns and after eliminating intercompany sales. Revenue is recognized when title, risks and rewards of the products are transferred to customers.

### *Revenue from out-licensing*

Out-licensing agreements are concluded with third parties, where the counterparty has to pay license fees. In situations where no further performance commitment exists, revenue is recognized on the earlier of when payments are received or collection is assured. Where continuous involvement for a certain period is required in the form of technology transfer or technical support, revenues are recognized over the period in question.

### *Revenue associated with up-front payments or performance milestones*

Such revenue is recognized in accordance with respective agreements.

### *Revenue from royalties*

Royalty payments are recognized on an accrual basis in accordance with the respective agreements.

### *Interest income*

Interest income is recognized on a pro rata temporis basis using the effective interest method.

## **Development / intangible assets**

Development expenses are charged to the income statement as incurred. They are capitalized as intangible assets when it is probable that future economic benefits will flow to Santhera. Such intangible assets are amortized on a straight-line basis over the period of the expected benefit when the asset becomes available for use, and are reviewed for impairment at each balance sheet date. Assets not available for use are tested annually.

### 3 Critical Accounting Estimates, Assumptions and Judgments

The preparation of financial statements in conformity with IFRS requires the use of certain critical accounting estimates. It also requires management to exercise its judgment in the process of applying Santhera's accounting policies. Santhera makes estimates and assumptions concerning the future. The resulting accounting will not necessarily equal the related actual outcome. The following areas involve assumptions and estimates that can have a significant impact on the consolidated financial statements:

- Measurement and impairment testing of intangible assets, see note 7 "*Impairment Test for Intangible Assets*".
- Measurement and impairment testing of inventory, see note 9 "*Inventories*".
- Personnel expenses from share-based payments in accordance with IFRS 2, i.e. estimates regarding the valuation of employee stock options when granted or modified, see note 17 "*Stock Option Plans*".
- Actuarial valuations in the context of defined benefit pension plans where various assumptions on e.g. discount rates, salary increase rates and mortality rates, etc. bear significant uncertainties due to the long-term nature of the plans, see note 21 "*Employee Expenses and Benefits*".

### 4 Exchange Rates of Principal Currencies

	Income statement in CHF average rates		Balance sheet in CHF year-end rates	
	2015	2014	2015	2014
1 Euro (EUR)	1.0681	1.2146	1.0826	1.2028
1 US dollar (USD)	0.9624	0.9152	0.9927	0.9895
1 British pound (GBP) <sup>1</sup>	1.4708	n/a	1.4694	n/a
1 Canadian dollar (CAD)	0.7534	0.8287	0.7157	0.8510

<sup>1</sup> Since annual reporting 2015.

## 5 Tangible Assets

	In CHF thousands	Equipment	IT hardware	Leasehold improvements	2015
<b>Cost</b>					
At January 1		185	334	42	561
Additions		61	286	3	350
Disposals		-22	-53	0	-75
Exchange differences		1	0	0	1
<b>At December 31</b>		<b>225</b>	<b>567</b>	<b>45</b>	<b>837</b>

### Accumulated depreciation and impairment losses

At January 1		177	211	41	429
Additions		11	73	1	85
Disposals		-22	-53	0	-75
Exchange differences		0	0	0	0
<b>At December 31</b>		<b>166</b>	<b>231</b>	<b>42</b>	<b>439</b>
<b>Net book value</b>		<b>59</b>	<b>336</b>	<b>3</b>	<b>398</b>

	In CHF thousands	Equipment	IT hardware	Leasehold improvements	2014
<b>Cost</b>					
At January 1		184	633	540	1,357
Additions		1	158	0	159
Disposals		-5	-458	-493	-956
Exchange differences		0	1	0	1
Reclassification		5	0	-5	0
<b>At December 31</b>		<b>185</b>	<b>334</b>	<b>42</b>	<b>561</b>

### Accumulated depreciation and impairment losses

At January 1		170	617	531	1,318
Additions		7	51	8	66
Disposals		-5	-458	-493	-956
Exchange differences		0	1	0	1
Reclassification		5	0	-5	0
<b>At December 31</b>		<b>177</b>	<b>211</b>	<b>41</b>	<b>429</b>
<b>Net book value</b>		<b>8</b>	<b>123</b>	<b>1</b>	<b>132</b>

## 6 Intangible Assets

In CHF thousands	Raxone/ Catena	Fipamezole	IT software/ patents	2015
<b>Cost</b>				
At January 1	30,387	3,918	312	<b>34,617</b>
Additions	0	0	165	<b>165</b>
<b>At December 31</b>	<b>30,387</b>	<b>3,918</b>	<b>477</b>	<b>34,782</b>
<b>Accumulated amortization and impairment losses</b>				
At January 1	26,157	3,918	268	<b>30,343</b>
Additions	1,013	0	24	<b>1,037</b>
Reversal impairment	-26,157	0	0	<b>-26,157</b>
<b>At December 31</b>	<b>1,013</b>	<b>3,918</b>	<b>292</b>	<b>5,223</b>
<b>Net book value</b>	<b>29,374</b>	<b>0</b>	<b>185</b>	<b>29,559</b>

In CHF thousands	Raxone/ Catena	Fipamezole	IT software/ patents	2014
<b>Cost</b>				
At January 1 (after correction)	30,387	3,918	292	<b>34,597</b>
Additions	0	0	47	<b>47</b>
Disposals	0	0	-28	<b>-28</b>
Exchange differences	0	0	1	<b>1</b>
<b>At December 31</b>	<b>30,387</b>	<b>3,918</b>	<b>312</b>	<b>34,617</b>
<b>Accumulated amortization and impairment losses</b>				
At January 1 (after correction)	26,157	3,918	287	<b>30,362</b>
Additions	0	0	9	<b>9</b>
Disposals	0	0	-28	<b>-28</b>
Exchange differences	0	0	0	<b>0</b>
<b>At December 31</b>	<b>26,157</b>	<b>3,918</b>	<b>268</b>	<b>30,343</b>
<b>Net book value</b>	<b>4,230</b>	<b>0</b>	<b>44</b>	<b>4,274</b>

<sup>1</sup> Some positions have been corrected, see note 2 "Correction of errors".

As a result of receiving the European marketing authorization in September 2015, Santhera determined the recoverable amount of its previously impaired intangible asset "Raxone/Catena". This resulted in a reversal of impairment of CHF 26.2 million which has been recorded under Development expenses (see note 7 "Impairment Testing of Intangible Assets").

## 7 Impairment Test for Intangible Assets

IAS 36 requires intangible assets not available for use to be tested for impairment on an annual basis by comparing the carrying value to its recoverable amount. The recoverable amount is the higher of fair value less costs of disposal and value in use. If there are indications that an impairment loss recognized in a previous period no longer exists or may have decreased, the recoverable amount of the asset or cash-generating unit is determined.

An entity should also consider the relationship between market capitalization and book values, among other factors, when reviewing for indicators of impairment. As of December 31, 2015, the market capitalization of Santhera was above the book value of its equity and therefore not indicating a potential impairment of the intangible assets.

Raxone/Catena forms the basis of the Raxone/Catena development projects. The intangible asset was previously impaired in 2012.

Santhera's main intangible asset was not available for use at the beginning of the reporting period and did not generate cash flows on a stand-alone basis. Based on the European marketing authorization, received in September 2015, an impairment test was performed which resulted in the full reversal of the previous impairments and an increase in the carrying amount of the intangible asset to its recoverable amount of CHF 29.4 million. At the same time the intangible asset formerly not available for use was transformed into an asset available for use with a finite useful life of 10 years. Amortization of the asset began in September 2015.

Management used the risk-adjusted Net Present Value (**rNPV**) model taking into consideration the expected cumulative probability of reaching the market to calculate recoverable amount. This is a customary model for the valuation of pharmaceutical intangibles. The rNPV model considers the net cash flows over the expected lifetime of the products based on the lifetime of the underlying intellectual property or the market exclusivity granted through orphan drug protection. For the purpose of estimating these cash flows, Santhera made estimates about the expected revenues based on estimated market size and patient numbers, expected market penetration rates, product pricing and project- or product-related costs. Santhera's strategic focus is on LHON and DMD. Since LHON is the most advanced program with a market authorization in the EU, received in 2015, the impairment test for 2015 is entirely based on project cash flows derived from this program in Europe (equal treatment end of 2014).

The key assumptions for the tests were as follows:

	<b>2015</b>	<b>2014</b>
Discount rate (WACC)	15%	15%
Market growth rate (terminal value)	0%	0%
Probability of reaching market	100%	>50%
Period of projected cash flows	5 years	5 years

## 8 Prepaid Expenses and Accrued Income

	In CHF thousands	2015	2014
Prepayments		1,467	47
Accrued income		0	323
Other accruals		46	6
<b>Total at December 31</b>		<b>1,513</b>	<b>376</b>

## 9 Inventories

	In CHF thousands	2015	2014
Raw material (active pharmaceutical ingredients)		1,552	0
Semi-finished goods		1,551	0
Finished goods		338	0
<b>Total at December 31</b>		<b>3,441</b>	<b>0</b>

Due to the marketing authorization received in 2015 for LHON in the EU, a reversal of impairment in the amount of TCHF 947 was recognized under development expenses (TCHF 164 less than reported in the interim reporting 2015 due to reconsiderations).

## 10 Trade and Other Receivables

	In CHF thousands	2015	2014
Trade receivables		1,466	610
Other receivables		665	110
<b>Total at December 31</b>		<b>2,131</b>	<b>720</b>

Trade receivables in 2015 mainly result from product sales, see note 18 *"Segment and Geographic Information"*. Other receivables consist mainly of amounts due from the government for tax reimbursements (e.g. VAT). They are due within 30 to 120 days and bear no interest. No allowance for doubtful debts was recognized on the receivables as management estimates that no allowance is necessary as of December 31, 2015, and 2014.

## 11 Cash and Cash Equivalents

	In CHF thousands	2015	2014
Cash at banks and on hand			
In CHF		69,570	16,416
In EUR		6,270	724
In GBP		772	0
In USD		191	267
In CAD		56	28
<b>Total at December 31</b>		<b>76,859</b>	<b>17,435</b>
<hr/>			
Short-term money market deposits			
In CHF		45,000	10,002

## 12 Share Capital

### Ordinary share capital

As of January 1, 2014, the share capital amounted to CHF 3,934,049, divided into 3,934,049 Shares at a nominal value of CHF 1 each. During 2014, 197,126 Shares were issued from conditional capital upon the exercise of stock options under the EIP, BSOP 2011, ESOP 2004, ESOP 2008 and ESOP 2010. 355,000 additional Shares were issued from conditional capital under the Standby Equity Distribution Agreement (**SEDA**) (see below). 288,317 Shares were issued from authorized capital for a private placement and 200,000 Shares were issued from conditional capital for sale by an independent broker. As a result, as of December 31, 2014, the share capital amounted to CHF 4,974,492, divided into 4,974,492 Shares at a nominal value of CHF 1 each.

During 2015, 398,306 Shares were issued from conditional capital upon the exercise of stock options. 590,000 Shares were issued from authorized capital for a private placement (accelerated bookbuilding) and 300,000 Shares were issued from conditional capital for sale by an independent broker. As a result, as of December 31, 2015, the share capital amounted to CHF 6,262,798, divided into 6,262,798 Shares at a nominal value of CHF 1 each.

### *Standby Equity Distribution Agreement*

In October 2013, Santhera entered into a SEDA with Yorkville Advisors Global Master SPV Ltd., New York, US (**YA Global**). Under the terms of the agreement, YA Global has committed to provide up to CHF 10 million in equity financing during a period of three years. The SEDA has been established in order to support the funding of Santhera's operations. It remains at the sole discretion of the Company to determine the timing of the funding. During 2015 no draws were made. During 2014, Santhera drew a total of CHF 1.4 million from YA Global for which 399,425 Shares were delivered. The remaining amount for equity financing with YA Global amounts to CHF 8.1 million.

*Treasury shares*

In connection with the liquidation of Oy Juvantia Pharma, Turku, Finland (**Juvantia**), a company acquired in 2009, Santhera received 8,028 Shares from former Juvantia shareholders. These treasury shares serve as pledge from the former owners of Juvantia for compensation of a potential tax claim related to pre-acquisition activities of Juvantia. Final tax assessment by the Finnish authorities is expected to be obtained mid- to end 2016.

**Authorized share capital**

On the occasion of the ASM on May 11, 2015, the shareholders approved an extension of the authorized share capital of the Company. The Board is authorized to increase the share capital at any time until May 11, 2017, through the issuance of up to 1,500,000 Shares with a nominal value of CHF 1 each.

On December 2, 2015, 590,000 Shares were issued in an accelerated bookbuilding process. As a result, as of December 31, 2015, the Board is authorized to increase the share capital at any time until May 11, 2017, through the issuance of up to 910,000 Shares with a nominal value of CHF 1 each. An increase in partial amounts is permitted. For each such increase, the Board has to determine the issue price, the type of payment, the date of issuance of new Shares, the conditions for the exercise of pre-emptive rights and the beginning date for dividend entitlement.

**Conditional share capital**

At the ASM on May 11, 2015, the shareholders additionally approved a maximum increase of the share capital by an aggregate amount of CHF 800,000 (2014: CHF 800,000) through the issuance of a maximum of 800,000 (2014: 800,000) Shares with a nominal value of CHF 1 each. The Shares can be issued through the exercise of option rights which are granted according to respective regulations of the Board. The exercise price of each option to be granted shall, at the full discretion of the Board, either equal (i) the weighted average share price during the three months preceding the grant for employees outside the US and Canada, or (ii) the closing price of the Share at the grant date.

In addition, the shareholders approved a maximum increase of the share capital by an aggregate amount of CHF 950,000 (2014: CHF 600,000) through the issuance of a maximum of 950,000 (2014: 600,000) Shares with a nominal value of CHF 1 per Share by the exercise of option and/or conversion rights which can be granted in connection with the issuance of bonds, similar obligations or other financial instruments by the Company or another Group company, and/or by the exercise of options which are granted by the Company or another Group company. In the case of the issue of bonds, similar obligations or other financial instruments linked with option and/or conversion rights, and in the case of the issue of option rights, the pre-emptive right of shareholders is excluded.

As of December 31, 2015, the Company had a conditional share capital, pursuant to the above provisions, whereby the share capital may be increased by

- a maximum amount of CHF 401,694 (2014: CHF 604,029) through the issuance of up to 401,694 (2014: 604,029) Shares, under the exclusion of shareholders' pre-emptive rights, for option rights being exercised under the Company's stock option plans, see note 17 "*Stock Option Plans*", and
- a maximum amount of CHF 650,000 (2014: CHF 600,000) by issuing up to 650,000 (2014: 600,000) Shares, through the exercise of warrants/options and/or notes granted in connection with bonds or similar debt instruments linked with option and/or conversion rights granted by the Company.

## 13 Deferred Taxes

### Net deferred taxes recorded

	In CHF thousands	2015	2014
Temporary differences on inventory		3,061	0
<b>Deferred tax assets recognized</b>		<b>3,061</b>	<b>0</b>
<hr/>			
Temporary differences on intangible assets		5,167	831
Tax loss carryforwards		-5,167	-831
<b>Deferred tax liabilities recognized</b>		<b>0</b>	<b>0</b>
<hr/>			
Tax loss carryforwards		269,696	317,170
Of which recorded		-25,834	-4,153
<b>Of which unrecorded</b>		<b>243,862</b>	<b>313,017</b>
<hr/>			
Expiring in			
1 year		9,738	47,276
2 years		5,832	9,738
3 years		22,671	5,832
4 years		177,282	22,671
5 years		0	188,257
More than 5 years		0	11,265
Without expiration		28,339	27,978
<b>Total unrecorded tax loss carryforwards</b>		<b>243,862</b>	<b>313,017</b>

Due to the uncertainty surrounding the future results of operations and the uncertainty as to whether Santhera can use the loss carryforwards for tax purposes, deferred tax assets on tax loss carryforwards were only considered to the extent that they offset taxable temporary differences within the same taxable entity. As there are no temporary differences associated with investments in subsidiaries, no deferred tax liability has to be recognized. No deferred tax assets are calculated on temporary differences related to pension obligations from IAS 19 (TCHF 3,957 per December 31, 2015, and TCHF 2,680 per December 31, 2014, respectively).

## 14 Trade and Other Payables

	In CHF thousands	2015	2014
Trade payables		3,290	1,654
Other payables (nonfinancial)		376	512
<b>Total at December 31</b>		<b>3,666</b>	<b>2,166</b>

All positions are noninterest-bearing and usually settled within 30 to 60 days.

## 15 Accrued Expenses

	In CHF thousands	2015	2014
Development programs		700	422
Liabilities to employees		905	137
Accruals for pricing and reimbursement		673	0
Accrued marketing and sales expenses		671	38
Expenses for audit, consulting and other		333	264
<b>Total at December 31</b>		<b>3,282</b>	<b>861</b>

## 16 Commitments and Contingent Liabilities

### Commitments

*Commitment for operating lease (non-cancellable)*

	In CHF thousands	2015	2014
Within 1 year		398	125
After 1 year through to 5 years		278	0
After 5 years		15	0
<b>Total at December 31</b>		<b>691</b>	<b>125</b>

### Contingent liabilities

*Collaboration and license agreement with Takeda*

In September 2013, Santhera announced an agreement with Takeda Pharmaceutical Company Ltd, Osaka, Japan (**Takeda**) to license back all previously granted rights in DMD and FA in order to increase its strategic flexibility. In return, Takeda is eligible to obtain a percentage from future licensing and/or sales income generated by Santhera in DMD of up to EUR 7.0 million. In addition, Santhera has obtained the right to cross-reference Takeda's *idebenone* data for regulatory use in any indication and in any territory. If Santhera makes use of such cross-reference right, Takeda is eligible to obtain a percentage from future licensing and/or sales income generated by Santhera in such indications of up to EUR 3.0 million. Lastly, both companies agreed to terminate a similar agreement for FA signed in 2005 and Santhera's contingent liability of EUR 1.0 million payable to Takeda has been waived.

Takeda is eligible to receive up to EUR 1.0 million as a percentage from future income generated by Santhera to offset this waiver.

#### *Agreement with the University of Leuven*

In March 2005, Santhera entered into an agreement with Katholieke Universiteit Leuven, Leuven, Belgium (**KU Leuven**), under which KU Leuven assigned to Santhera its patents and patent applications relating to the use of Raxone/Catena to treat various forms of muscular-dystrophy-related disorders, particularly DMD. Based on this agreement, Santhera has filed patent applications in major territories covering the use of Raxone/Catena for the treatment of DMD.

KU Leuven is entitled to a success fee of up to EUR 0.4 million if and when Santhera commercializes any product in a major market, which includes the EU, the US or Japan and certain countries within the EU. In addition, in the event Santhera commercializes the product itself, KU Leuven is entitled to receive 5% royalties on net sales. In the event Santhera grants commercialization rights to a third party, KU Leuven will receive 15% of all the consideration received by Santhera from such third party.

#### *License agreement with Novartis*

On June 30, 2007, Santhera entered into an agreement with Novartis Pharma AG, Basel, Switzerland (**Novartis**), under which it in-licensed *omigapil*. Santhera develops *omigapil* for the treatment of Congenital Muscular Dystrophies (**CMD**). Additional payments will be due to Novartis a) upon start of a pivotal clinical trial, b) upon regulatory approval in a major market country, and c) after reaching certain commercialization milestones. Santhera will also have to pay royalties to Novartis calculated on net sales.

#### *Agreement with the National Institutes of Health*

In June 2013, Santhera has obtained an exclusive license from the National Institutes of Health, Bethesda/Maryland, US (**NIH**), to its rights on a patent granted in the US for the use of *idebenone* for the treatment of primary progressive Multiple Sclerosis. Under the terms of the agreement, Santhera would have to make certain milestone payments to the NIH not exceeding USD 1.4 million in total. Furthermore, the NIH is eligible to a royalty fee of 3% on net sales and 15% of considerations received in case Santhera sublicenses the program.

#### *Contracts for clinical development and other*

As part of its ordinary course of business, Santhera has entered into several contracts for e.g. clinical or technical development services. Commitments are within current market prices and can be terminated at the Company's discretion.

In order to meet its requirements for market supply, potential launch and inventory risk management purposes (security stock), Santhera entered into commitments for the purchase of active pharmaceutical ingredients in the amount of up to EUR 6.3 million (to be delivered in 2016).

### **Contingent liabilities summary**

Santhera believes that the disclosures above and accruals (see note 15 "*Accrued Expenses*") are adequate based upon currently available information. However, given the inherent difficulties in estimating liabilities relating to clinical development, regulatory, tax, possible litigation and certain other matters due to uncertainty concerning both the amount and timing of future expenditures, it cannot be guaranteed that additional costs will not be incurred materially beyond the amounts accrued.

## 17 Stock Option Plans

Santhera has established stock option plans to align the long-term interests of the members of the Board, the Executive Management and employees. Options granted under the stock option plans are equity-settled.

### Executive Incentive Plan (EIP)

In November 2006, under the EIP, the members of the Executive Management were granted stock options to acquire 101,065 Shares, as a management incentive. Each of these stock options entitles its holder to purchase one Share at an exercise price of CHF 1. The vesting period of the options was one year. At the end of the option term, i.e. after a period of ten years as from the grant date, all unexercised stock options will expire without value. The EIP is administered under the responsibility of the Board. No further grants can be made under the EIP.

#### *Options outstanding, exercised or forfeited under the EIP*

Number of options				2015				2014
Plan	Exer- cised	Forfeit- ed	Expired	Out- standing	Exer- cised	Forfeit- ed	Expired	Out- standing
EIP	790	0	0	1,210	42,598	0	0	2,000

### Employee Stock Option Plans

The Company adopted the ESOP 2004, ESOP 2008, ESOP 2010 and ESOP 2015 (collectively the **ESOPs**) to provide incentives to members of the Board, the Executive Management and employees helping to ensure their commitment to Santhera over the long term. Since January 1, 2015, new grants have been allocated under the ESOP 2015. Option grants are made from time to time at the discretion of the Board or as contractually agreed with employees. The ESOPs contain customary provisions in respect of the adjustment or cancellation of stock options upon termination of employment, retirement, death, disability and certain corporate transactions. All stock option plans are administered under the responsibility of the Board. Each stock option entitles its holder to purchase one Share of the Company at an exercise price defined to be either a) equal to the volume-weighted average share price in the three preceding months for Swiss employees, or b) the closing share price on the SIX Swiss Exchange (**SIX**) at each grant date. In general, 50% of the stock options vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. At the end of the option term, i.e. after a period of 10 years as from the grant date, unexercised stock options expire without value. Subject to the provisions of the ESOP 2004, vested stock options of employees leaving the Company in good faith do not lapse. Under the ESOP 2008 and ESOP 2010 vested stock options of employees leaving the Company in good faith expire six months after the termination date of the employment. Under the ESOP 2015 vested stock options of employees leaving the Company in good faith do not expire. Unvested stock options of employees leaving the Company are forfeited under all stock option plans.

*Options outstanding, exercised, forfeited or expired under ESOPs*

Number of options	2015				
	Exercised	Granted	Forfeited	Expired	Outstanding
ESOP 2004	9,045	0	0	0	26,091
ESOP 2008	0	0	0	0	1,500
ESOP 2010	358,971	0	2,700	0	47,773
ESOP 2015	0	142,260	2,000	0	140,260
<b>Total</b>	<b>368,016</b>	<b>142,260</b>	<b>4,700</b>	<b>0</b>	<b>215,624</b>

Number of options	2014				
	Exercised	Granted	Forfeited	Expired	Outstanding
ESOP 2004	45,834	0	0	4,365	35,136
ESOP 2008	4,000	0	0	0	1,500
ESOP 2010	70,694	332,800	5,800	850	409,444
<b>Total</b>	<b>120,528</b>	<b>332,800</b>	<b>5,800</b>	<b>5,215</b>	<b>446,080</b>

**Board Stock Option Plans**

The Company adopted the BSOP 2011 and BSOP 2015 (collectively the **BSOPs**) to provide incentives to members of the Board. Since January 1, 2015, new grants have been made under the BSOP 2015. The plan contains the same customary provisions as under the ESOP plans described above. Each stock option entitles its holder to purchase one Share of the Company at an exercise price defined to be either a) equal to the volume-weighted average share price in the three preceding months, or b) the closing share price on the SIX at each grant date. In general, 50% of the stock options vest on the second anniversary, 25% on the third anniversary and the remaining 25% on the fourth anniversary of the grant date. At the end of the option term, i.e. after a period of 10 years as from the grant date, unexercised stock options expire without value. Under the BSOP 2011 vested stock options of Board members leaving the Board in good faith expire six months after the termination date of them being a member of the Board while unvested stock options of Board members leaving the Board in good faith are forfeited. Under the BSOP 2015 vested and unvested stock options of Board members leaving the Board in good faith do not expire.

*Options outstanding, exercised, forfeited or expired under BSOPs*

Number of options	<b>2015</b>				
	Exercised	Granted	Forfeited	Expired	Outstanding
BSOP 2011	29,500	0	0	0	0
BSOP 2015	0	7,000	0	0	7,000
<b>Total</b>	<b>29,500</b>	<b>7,000</b>	<b>0</b>	<b>0</b>	<b>7,000</b>

Number of options	<b>2014</b>				
	Exercised	Granted	Forfeited	Expired	Outstanding
BSOP 2011	34,000	29,500	0	0	29,500

As of December 31, 2015, 177,860 stock options (2014: 126,449) are available for future grants under the ESOP 2015 and/or the BSOP 2015.

**Fair value calculations for stock options granted**

The fair value of stock options is determined at each grant date by using the Hull-White option pricing model. The calculation of the option value was performed by applying the following parameters:

	<b>2015</b>	<b>2014</b>
Market price of stock	CHF 80.20 to 138.90	CHF 3.46 to 104.90
Exercise prices	CHF 83.00 to 133.08	CHF 4.23 to 101.00
Weighted average fair value of options granted	CHF 40.12	CHF 2.93
Expected volatility <sup>1</sup>	43% to 46%	50% to 53%
CHF risk-free interest rate	-0.10% to 0.38% p.a.	0.71% to 0.98% p.a.
Option term <sup>2</sup>	10 years	10 years
Expected dividend yield	0%	0%

<sup>1</sup> The expected volatility was determined on the basis of selected biotech companies.

<sup>2</sup> After expiration of the vesting period, the stock options become American-style options and may be exercised any time until the end of the option term. The option pricing model takes into consideration certain assumptions about potential early exercises.

**Number of stock options outstanding and exercisable**

	Number of options	<b>2015</b>	<b>2014</b>
<b>Outstanding at January 1</b>		<b>477,580</b>	<b>323,421</b>
Granted		149,260	362,300
Exercised <sup>1</sup>		-398,306	-197,126
Forfeited		-4,700	-5,800
Expired		0	-5,215
<b>Outstanding at December 31</b>		<b>223,834</b>	<b>477,580</b>
<b>Exercisable at December 31</b>		<b>60,412</b>	<b>98,655</b>

<sup>1</sup> The average closing share price of options exercised during the reporting period 2015 was CHF 95.40 (2014: CHF 47.40).

The value of stock options granted is recognized as personnel expense over the period Santhera receives services. In 2015, stock option grants resulted in personnel expenses of TCHF 1,528 (TCHF 277 related to Development, TCHF 580 related to Marketing & Sales (M&S) and TCHF 671 to General & Administration (G&A)) and in 2014, such grants resulted in personnel expenses of TCHF 759 (TCHF 386 related to Development, TCHF 26 related to M&S and TCHF 347 to G&A). In the first quarter of 2016, Santhera allocated 90,730 stock options which form part of the bonus award to employees for the year ended December 31, 2015. Although these stock options were not legally granted in 2015, Executive Management considers it appropriate to recognize expenses in 2015 as employees have been rendering services in 2015 in expectation of the annual bonus allocation. Personnel expenses in 2015 for this amounted to TCHF 512 (TCHF 108 related to Development, TCHF 244 related to M&S and TCHF 160 related to G&A). In the first quarter of 2015, Santhera allocated 39,760 stock options which formed part of the bonus award to employees for the year ended December 31, 2014. Personnel expenses in 2014 for this amounted to TCHF 418 (TCHF 181 related to Development, TCHF 6 related to M&S and TCHF 231 related to G&A) (see note 2 "Correction of errors").

In January 2014, a total of 352,000 options with exercise prices between CHF 3.78 and CHF 4.02 were granted. The majority of these options were exceptionally granted in order to reduce the risk of losing employees at a time when the Company was in a very critical financial situation.

#### Terms of options outstanding at December 31

Exercise price range for options (in CHF)	Number outstanding	Weighted average remaining contractual life (years)	2015 Number exercisable	Number outstanding	Weighted average remaining contractual life (years)	2014 Number exercisable
1.00	1,210	0.86	1,210	2,000	1.85	2,000
from 3.78 to 6.34	42,673	7.44	31,611	428,644	8.75	60,019
from 22.25 to 30.10	6,600	7.43	1,500	11,800	8.90	1,500
from 59.44 to 60.25	19,788	0.52	19,788	28,833	1.62	28,833
from 82.58 to 114.50	153,563	9.02	6,303	6,303	1.89	6,303
<b>Total</b>	<b>223,834</b>	<b>7.87</b>	<b>60,412</b>	<b>477,580</b>	<b>8.20</b>	<b>98,655</b>

## 18 Segment and Geographic Information

### Segment information

Santhera operates in one operating segment, the development and commercialization of specialty niche products for the treatment of mitochondrial and neuromuscular diseases. The Board, the Executive Management and senior managers, being the CODM, assess the reporting data and allocate resources as one segment on an aggregated consolidated level according to the operating expenses by function. Santhera generates revenue from sales of Raxone/Catena for the treatment of LHON, DMD and FA. Geographic revenue information is based on location of the customer.

## Geographic information

### *Net sales*

	In CHF thousands	2015	2014
EU		4,321	2,548
Rest of the world		0	43
<b>Total</b>		<b>4,321</b>	<b>2,591</b>

In 2015, net sales of Raxone/Catena were generated after European marketing authorization in LHON and under special programs (e.g. the French temporary authorization for use as well as international Named Patient Programs) in the amount of CHF 4.3 million. In 2014 net sales of Raxone/Catena amounted to CHF 2.6 million, mainly in the EU.

### *Noncurrent assets (excluding financial instruments and deferred taxes)*

	In CHF thousands	2015	2014
			<b>Restated</b>
Switzerland		29,876	4,403
EU		80	0
North America		1	3
<b>Total</b>		<b>29,957</b>	<b>4,406</b>

## 19 Other Operating Income

This position consists primarily of reimbursements from scientific programs.

## 20 Operating Expenses by Nature

	In CHF thousands	2015	2014
			<b>Restated</b>
External Development expenses		-6,341	-3,168
Reversal of impairment of intangible asset		26,157	0
Reversal of impairment on inventories		947	0
Patent and license expenses		-222	-229
Marketing expenses		-3,870	-446
Employee expenses		-13,105	-5,165
Of which non-cash-relevant expenses for share-based payments		-2,040	-1,177
Other administrative expenses		-2,999	-1,517
Depreciation, amortization and impairment		-110	-75
Lease expenses		-406	-251
Other operating expenses		-16	-9
<b>Total operating expenses</b>		<b>35</b>	<b>-10,860</b>

## 21 Employee Expenses and Benefits

### Employee expenses

	In CHF thousands	2015	2014 Restated
Wages and salaries		-6,435	-3,002
Social security and other personnel-related expenses <sup>1</sup>		-4,630	-986
Of which non-cash-relevant adjustments of pension fund		394	9
Share-based payments		-2,040	-1,177
<b>Total employee costs</b>		<b>-13,105</b>	<b>-5,165</b>
<hr/>			
<b>Average number of full-time equivalents<sup>2</sup></b>		<b>31.4</b>	<b>13.8</b>
<hr/>			
<b>Full-time equivalents at year-end</b>		<b>53.3</b>	<b>14.7</b>
<hr/>			
<b>Total headcount at year-end</b>		<b>59</b>	<b>18</b>

<sup>1</sup> Thereof TCHF 18 were expensed for defined contribution plans in North America and some EU countries (2014: TCHF 3).

The increase to the previous period results from higher social security expenses on option exercises.

<sup>2</sup> For the calculation of full-time equivalents, only employees with part-time and full-time permanent working contracts are taken into consideration.

### Termination benefits

In 2015 and 2014, no termination benefits were expensed.

### Pension plan

In accordance with the Swiss pension fund law "Federal Act on Occupational Old Age, Survivors' and Invalidity Pension Provision" (OPA), all employees of Santhera Pharmaceuticals Holding AG and Santhera Pharmaceuticals (Schweiz) AG, both in Liestal, Switzerland, have to be affiliated with a collective independent pension fund. These funds provide for retirement benefits, as well as risk benefits (death and disability). The plans qualify as defined benefit plans under IAS 19 and the assets cannot revert to the employer. Contributions to the plans are such that the employee contributes 40% and the employer the rest. Contributions are computed as percentage of the salary, depending on age. In order to manage these risks, Santhera entered into an agreement with AXA Foundation for occupational benefits (AXA foundation). The AXA foundation is responsible for the governance of the plan; the board is composed of an equal number of representatives from the employers and employees chosen from all affiliated companies. AXA foundation has set up investment guidelines, defining in particular the strategic allocation with margins. AXA foundation has reinsured its risks (investment risk, mortality risk, etc.) with AXA Life Ltd, Winterthur, Switzerland (AXA). AXA manages the savings capital/investments on behalf of AXA foundation. The accumulated savings capital is allocated to each insured individual and consists of annual contributions, savings credits and interest credits. In certain situations, additional payments or increased periodic contributions by the employer may become due based on the pension plans funded status as measured under Swiss pension rules (OPA).

An independent actuary has performed the respective calculations as required by IAS 19:

*Changes in defined benefit obligations*

	In CHF thousands	2015	2014
<b>Present value of obligation, January 1</b>		<b>7,747</b>	<b>4,176</b>
Current employer service cost		704	297
Past service cost <sup>1</sup>		-656	-89
Interest cost		76	94
Employee contributions		267	138
Benefits paid / transfer payments		6,074	1,524
Insurance premiums		-142	-75
Remeasurements <sup>2</sup>		1,727	1,682
<b>Present value of obligation, December 31</b>		<b>15,797</b>	<b>7,747</b>

<sup>1</sup> Decrease of obligation due to reduction of the conversion rates for the over-mandatory part of the retirement capital.

<sup>2</sup> Details of remeasurements:

	In CHF thousands	2015	2014
Actuarial (gain)/loss due to changes in financial assumptions		170	1,604
Actuarial (gain)/loss due to experience adjustments		1,557	78
<b>Subtotal (gain)/loss</b>		<b>1,727</b>	<b>1,682</b>
(Return)/loss on plan assets (excluding interest income)		-56	10
<b>Total remeasurements in other comprehensive income (gain)/loss</b>		<b>1,671</b>	<b>1,692</b>

*Changes in plan assets*

	In CHF thousands	2015	2014
<b>Fair value of assets, January 1</b>		<b>5,067</b>	<b>3,179</b>
Interest income on assets		55	77
Employer contributions		463	234
Employee contributions		267	138
Benefits paid / transfer payments		6,074	1,524
Insurance premiums		-142	-75
Remeasurements (return/(loss) on plan assets (excluding interest income))		56	-10
<b>Fair value of assets, December 31</b>		<b>11,840</b>	<b>5,067</b>

*Net defined benefit asset/(obligation)*

	In CHF thousands	2015	2014
Present value of obligation, December 31		15,797	7,747
Fair value of assets, December 31		11,840	5,067
<b>Net defined asset/(obligation)</b>		<b>-3,957</b>	<b>-2,680</b>

*Asset breakdown*

Assets of the defined benefit plan are not quoted since AXA fully insures them. Therefore the entire amount of TCHF 9,499 (2014: TCHF 5,067) is treated as an insurance contract and has no quoted market price.

*The weighted-average assumptions to determine benefit obligations and defined benefit cost were as follows:*

	In %	2015	2014
Discount rate		0.90	1.05
Expected future salary increases		1.50	1.50

*Sensitivity analysis for 2015:*

In CHF thousands	Defined benefit obligation		Gross service cost	
	Increase assumption	Decrease assumption	Increase assumption	Decrease assumption
Discount rate +/-0.25%	-537	578	-73	78
Salary increase +0.25%	84	-	-1	-
Live expectancy +1 year	245	-	20	-

*Sensitivity analysis for 2014:*

In CHF thousands	Defined benefit obligation		Gross service cost	
	Increase assumption	Decrease assumption	Increase assumption	Decrease assumption
Discount rate +/-0.25%	-369	397	-31	33
Salary increase +0.25%	73	-	-4	-
Live expectancy +1 year	150	-	10	-

*Mortality rate:*

Life expectancy at age 65	2015	2014
Male	21.59	21.49
Female	24.06	23.96

The expected employer contributions for fiscal year 2016 amount to approximately TCHF 568 (2014: TCHF 256). No benefit obligations for pensioners exist at December 31, 2015 (2014: none). The duration of the plan liabilities calculated is 20.8 years as per December 31, 2015 (2014: 20.8 years).

## 22 Financial Income/Expenses

### Financial income

	In CHF thousands	2015	2014
Interests on cash and cash equivalents		2	4
Realized and unrealized foreign exchange gains		414	50
<b>Total</b>		<b>416</b>	<b>54</b>

### Financial expenses

	In CHF thousands	2014	2014
Interest expenses		-11	-7
Realized and unrealized foreign exchange losses		-644	-62
<b>Total</b>		<b>-655</b>	<b>-69</b>

## 23 Income Taxes

	In CHF thousands	2015	2014 Restated
Current income tax income/(expense)		-46	-2
Deferred tax income/(expense)		3,061	0
<b>Total</b>		<b>3,015</b>	<b>-2</b>

The following is a theoretical reconciliation of the income taxes calculated at the Group's expected effective income tax rate:

	In CHF thousands	2015	2014
Result before taxes		2,934	-7,950
Tax (expense)/income at expected group tax rate of 20% <sup>1</sup>		-587	1,590
Effect of tax rate difference group versus local		-2,413	0
Effect of non-deductible expenses		-12	0
Utilization of previously unrecognized tax losses		7,376	0
Recognition of DTA on previously unrecognized tax losses		4,336	0
Unrecognized deferred taxes		-5,685	-1,592
Effective tax income/(expense)		3,015	-2

<sup>1</sup> The tax rate of 20% represents the Group's expected long-term tax rate based on rates applicable in those jurisdictions where taxable income should be generated in the future.

According to currently applicable Swiss tax law, the period to offset tax loss carryforwards against taxable profit is limited to seven years. According to currently applicable German tax law, tax loss carryforwards can, besides other conditions, be offset against taxable profit for an unlimited period but only to an amount of EUR 1.0 million and in addition for 60% of further amounts beyond this threshold per annum.

## 24 Earnings/Loss per Share

Basic earnings/loss per share is calculated by dividing the net profit/net loss attributable to equity holders by the weighted average number of Shares issued and outstanding during the reporting period, excluding Shares held as treasury shares (purchased at market).

	2015	2014 Restated
Net result attributable to shareholders (in CHF)	5,949,239	-7,951,925
Weighted average number of shares issued and outstanding	5,343,089	4,704,000
<b>Basic net result per share (in CHF)</b>	<b>1.11</b>	<b>-1.69</b>

Diluted earnings per share are calculated by dividing the net profit attributable to owners of ordinary Shares of the Company by the weighted average number of Shares issued and outstanding during the reporting period adjusted for Shares held as treasury shares (purchased at market) and the number of potential shares from stock option plans. For 2014 no diluted net result was calculated since the exercise of stock options would have been anti-dilutive.

	2015	2014
Net result attributable to shareholders (in CHF)	5,949,239	n/a
Weighted average number of shares issued and outstanding	5,343,089	n/a
Additional shares of potential option exercise	140,441	n/a
Adjusted weighted average number of shares issued and outstanding	5,483,530	n/a
<b>Diluted net result per share (in CHF)</b>	<b>1.08</b>	<b>n/a</b>

## 25 Related Party Transactions

### Board and Executive Management compensation

#### *Total compensation of Board and Executive Management*

	In CHF thousands	2015	2014 Restated
Compensation, wages and salaries		2,043	504
Post-employment benefits (pension fund contributions)		211	35
Share-based payment expenses (fair value according to IFRS 2)		855	431

#### *Transactions with members of the Board and Executive Management*

There are no loans outstanding or guarantee commitments granted to members of the Board and Executive Management.

In 2015, 29,500 stock options were exercised by members of the Board (2014: 24,000 stock options exercised). 211,394 stock options were exercised by the Executive Management (2014: 23,895 stock options exercised).

## 26 Risk Management Objectives and Policies

Santhera Pharmaceuticals Holding AG maintains a Group-wide corporate risk management system consisting of the areas corporate governance, financial internal controls and quality control / quality assurance.

On a regular basis, operational corporate risks are identified and their likelihood and impact assessed (gross risks). By defining and undertaking appropriate measures, these risks are managed accordingly to either reduce or avoid such risk (net risk). The results of this process are discussed at Board meetings.

Those risks as identified within the area of accounting and financial reporting as well as related control processes are further covered by the Company's Group-wide internal control system.

Santhera conducts development activities primarily in Switzerland, the EU and the US and is exposed to a variety of financial risks, such as, but not limited to, foreign exchange rate risk, credit risk, liquidity risk, cash flow and interest rate risk. Part of Santhera's overall risk management focuses on financial risks and the unpredictability of financial markets seeking to minimize potential adverse effects on the financial performance of the Group. Special guidelines and policies approved by the Board exist for overall risk management, financial internal controls and treasury management and are monitored by the Executive Management and the Board on a regular basis. The risk of foreign exchange rate fluctuations on the expenses can partly be managed by entering into foreign exchange derivative contracts. In accordance with the relevant treasury guidelines, Santhera only concludes contracts with selected high-quality financial institutions of good reputation and is not allowed to engage in speculative transactions. In addition, Santhera's treasury guidelines currently limit the Company to engage in money market deposits or similar instruments with a maturity beyond 12 months.

### Foreign exchange rate risk

Santhera holds cash amounts in four major currencies CHF, EUR, USD, GBP and CAD to cover the majority of future expected expenses. In addition, in order to reduce its foreign exchange rate exposure, Santhera occasionally enters into derivative currency contracts (forwards, options, structured derivatives) to hedge against additional major foreign currency exchange rate fluctuations. Evaluations based on market values were performed regularly. Any fair value changes of such currency positions are recorded accordingly in the income statement. Santhera's primary exposure to financial risk is due to fluctuation of exchange rates between CHF, EUR, USD, GBP and CAD. No derivative currency contracts are outstanding as of December 31, 2015 and 2014.

The following table demonstrates the sensitivity to a reasonable possible change in the EUR exchange rate, with all other variables held constant, of the Group's result before taxes. There is no impact on the Group's equity.

	Increase/decrease foreign currency rate	Effect on result before taxes in CHF thousands
<b>EUR positions</b>		
<b>2015</b>	+10%	-608
	-10%	608
<b>2014</b>	+5%	-17
	-20%	67

### Interest rate risk

Santhera earns interest income on cash and cash equivalents and its profit and loss may be influenced by changes in market interest rates. Santhera is either holding its cash on deposit/current accounts or investing cash through money market instruments in line with its treasury guidelines to follow its financial needs over time.

The following table demonstrates the sensitivity to a reasonable change in interest rates, with all other variables held constant, of the Group's result before taxes. There is no impact on the Group's equity.

As per end of 2015, variances of +/-50 basis points were calculated, resulting in fluctuations of +/- TCHF 384 before tax (end of 2014: +/-50 basis points resulting in fluctuations of +/-TCHF 87 before tax).

### Credit risk

Santhera has a certain concentration of credit risk. Short-term investments are invested as cash on deposit or in low-risk money market funds, i.e. money market accounts with government-backed corporate banks, top-tier categorized banks or S&P A-1 rated money market investment instruments or similar ratings. No investment or contract with any single counterparty, except cash on deposit subject to the criteria above, comprises more than 20% of cash and cash equivalents at the date of investment.

Santhera has policies in place to ensure that sales of products or entered partnerships are made to or entered with customers or partners with an appropriate credit history and a commitment to ethical business practices. The maximum credit risk exposure is limited to the carrying amount of its financial assets including derivatives.

### Liquidity risk

Prudent liquidity risk management implies maintaining sufficient cash and cash equivalents. Currently, the Company is financed through equity and there is no interest-bearing funding through debt instruments. Santhera's treasury calculates on a rolling basis the needs for aligning the current expenses against the need for optimized financial investments.

*Contractual undiscounted cash flows*

<b>Year ended December 31, 2015</b> In CHF thousands	On demand	Less than 3 months	3 to 12 months	1 to 5 years	After 5 years	Total	Book value
Accrued expenses	0	2,609	0	0	0	2,609	2,609
Trade payables	0	3,291	0	0	0	3,291	3,291
<b>Total</b>	<b>0</b>	<b>5,900</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>5,900</b>	<b>5,900</b>

<b>Year ended December 31, 2014</b> In CHF thousands	On demand	Less than 3 months	3 to 12 months	1 to 5 years	After 5 years	Total	Book value
Accrued expenses	0	861	0	0	0	861	861
Trade payables	0	1,654	0	0	0	1,654	1,654
<b>Total</b>	<b>0</b>	<b>2,515</b>	<b>0</b>	<b>0</b>	<b>0</b>	<b>2,515</b>	<b>2,515</b>

**Categories of financial instruments**

<b>Year ended December 31, 2015</b> In CHF thousands	Book value	Loans and receivables	Other liabilities at amortized cost
<b>Assets</b>			
Financial assets long-term	190	190	0
Trade receivables	1,466	1,466	0
Other receivables	49	49	0
Cash and cash equivalents	76,859	76,859	0
<b>Total</b>	<b>78,564</b>	<b>78,564</b>	<b>0</b>
<b>Liabilities</b>			
Trade payables	3,291	0	3,291
<b>Total</b>	<b>3,291</b>	<b>0</b>	<b>3,291</b>

<b>Year ended December 31, 2014</b> In CHF thousands	Book value	Loans and receivables	Other liabilities at amortized cost
<b>Assets</b>			
Financial assets long-term	85	85	0
Trade receivables	610	610	0
Other receivables	41	41	0
Cash and cash equivalents	17,435	17,435	0
<b>Total</b>	<b>18,171</b>	<b>18,171</b>	<b>0</b>
<b>Liabilities</b>			
Trade payables	1,654	0	1,654
<b>Total</b>	<b>1,654</b>	<b>0</b>	<b>1,654</b>

### Capital management

The first priority of Santhera's capital management is to provide adequate cash funds to ensure the financing of successful development and marketing activities so that future profits can be generated by gaining marketing authorization approvals for pharmaceutical products. As a company with currently one product on a smaller market, the capital management continues to be focused on the cash and cash equivalents position and is governed by specific Group treasury guidelines.

The funds raised in various private financing rounds, the private placement in 2008, 2014 and 2015, SEDA, the sale of Shares by an independent broker as well as funds generated through product sales and revenue from licensing enabled the Group to be adequately financed.

No changes in goals and policies of the treasury management have been made during the past two reporting years.

## 27 Events After the Reporting Date

None

## Report of the Statutory Auditor on the Consolidated Financial Statements

Basel, April 11, 2016

As statutory auditor, we have audited the consolidated financial statements of Santhera Pharmaceuticals Holding AG, which comprise the consolidated balance sheet, consolidated income statement, consolidated statement of comprehensive income, consolidated cash flow statement, consolidated statement of changes in equity and notes (pages 12 to 49), for the year ended 31 December 2015.

### **Board of Directors' responsibility**

The Board of Directors is responsible for the preparation of these consolidated financial statements in accordance with IFRS and the requirements of Swiss law and the consolidation and valuation principles as set out in the notes. This responsibility includes designing, implementing and maintaining an internal control system relevant to the preparation of consolidated financial statements that are free from material misstatement, whether due to fraud or error. The Board of Directors is further responsible for selecting and applying appropriate accounting policies and making accounting estimates that are reasonable in the circumstances.

### **Auditor's responsibility**

Our responsibility is to express an opinion on these consolidated financial statements based on our audit. We conducted our audit in accordance with Swiss law and Swiss Auditing Standards and International Standards on Auditing. Those standards require that we plan and perform the audit to obtain reasonable assurance whether the consolidated financial statements are free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the consolidated financial statements. The procedures selected depend on the auditor's judgment, including the assessment of the risks of material misstatement of the consolidated financial statements, whether due to fraud or error. In making those risk assessments, the auditor considers the internal control system relevant to the entity's preparation of the consolidated financial statements in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the entity's internal control system. An audit also includes evaluating the appropriateness of the accounting policies used and the reasonableness of accounting estimates made, as well as evaluating the overall presentation of the consolidated financial statements. We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our audit opinion.

## **Opinion**

In our opinion, the consolidated financial statements for the year ended 31 December 2015 give a true and fair view of the financial position, the results of operations and the cash flows in accordance with IFRS and comply with Swiss law.

## **Report on other legal requirements**

We confirm that we meet the legal requirements on licensing according to the Auditor Oversight Act (AOA) and independence (article 728 CO and article 11 AOA) and that there are no circumstances incompatible with our independence.

In accordance with article 728a paragraph 1 item 3 CO and Swiss Auditing Standard 890, we confirm that an internal control system exists, which has been designed for the preparation of consolidated financial statements according to the instructions of the Board of Directors.

We recommend that the consolidated financial statements submitted to you be approved.

Ernst & Young Ltd

/s/ Jolanda Dolente  
Licensed audit expert  
(Auditor in charge)

/s/ Nicole Riggenbach  
Licensed audit expert

# Statutory Financial Statements of Santhera Pharmaceuticals Holding AG

## Contents

Balance Sheet .....	53
Income Statement .....	54
Notes to the Statutory Financial Statements .....	55
1 Introduction .....	55
2 Principles .....	55
3 Information on Balance Sheet and Income Statement Items .....	56
4 Other Information .....	58
Proposals of the Board of Directors to the Annual Shareholders' Meeting .....	62
Report of the Statutory Auditor on the Financial Statements .....	64

## Balance Sheet

	As of December 31, in CHF thousands	Notes	2015	2014
<b>Assets</b>				
Cash and cash equivalents			61,256	6,953
Other receivables from third parties			43	8
Other receivables from shareholdings			287	415
Prepaid expenses and accrued income			180	23
Treasury shares			0	177
<b>Current assets</b>			<b>61,766</b>	<b>7,576</b>
Loans to shareholdings		3.1	0	0
Investments in shareholding		3.2	59	0
<b>Noncurrent assets</b>			<b>59</b>	<b>0</b>
<b>Total assets</b>			<b>61,825</b>	<b>7,576</b>
<b>Liabilities and equity</b>				
Trade accounts payable to third parties			154	68
Other accounts payable to third parties			188	447
Other accounts payable to shareholdings			188	49
Accrued expenses			297	171
<b>Current liabilities</b>			<b>827</b>	<b>735</b>
<b>Total liabilities</b>			<b>827</b>	<b>735</b>
Share capital		3.3	6,263	4,974
<i>Reserves from capital contributions</i>			57,083	3,049
<i>Other capital reserves</i>			2,891	916
Statutory capital reserves			59,974	3,965
<i>Reserves for treasury shares</i>			0	177
Statutory retained earnings			0	177
<i>Accumulated result</i>			-5,557	-2,593
<i>Results carried forward</i>			-2,593	-1,503
<i>Net result for the period</i>			-2,964	-1,090
<i>Other voluntary reserves (free reserves)</i>			495	318
Voluntary accumulated result and other reserves			-5,062	-2,275
Treasury shares		3.4	-177	0
<b>Total equity</b>			<b>60,998</b>	<b>6,841</b>
<b>Total liabilities and equity</b>			<b>61,825</b>	<b>7,576</b>

## Income Statement

For the year ended December 31, in CHF thousands	Notes	2015	2014
Income from shareholdings	3.5	1,970	1,203
Other operating income		1	1
<b>Total operating income</b>		<b>1,971</b>	<b>1,204</b>
General and administrative expenses	3.6	-3,322	-1,463
Employee costs		-1,656	-845
Other operating expenses		-2	-2
<b>Total operating expenses</b>		<b>-4,980</b>	<b>-2,310</b>
<b>Operating result</b>		<b>-3,009</b>	<b>-1,106</b>
Financial income		21	31
Financial expenses		-35	-15
<b>Financial result</b>		<b>-14</b>	<b>16</b>
Reversal on allowance of investment		59	0
<b>Result before taxes</b>		<b>-2,964</b>	<b>-1,090</b>
Direct taxes		0	0
<b>Net result</b>		<b>-2,964</b>	<b>-1,090</b>

## Notes to the Statutory Financial Statements

### 1 Introduction

Santhera Pharmaceuticals Holding AG (the Company or Santhera) is the parent company of Santhera Group. The Company has its registered offices at Hammerstrasse 49 in 4410 Liestal, Switzerland.

### 2 Principles

#### General

Beginning in the year ended December 31, 2015, the statutory financial statements of the Company are prepared in accordance with the general accepted accounting principles as set out in art. 957 to art. 963b, of the Swiss Code of Obligations (CO), which became effective since January 1, 2013, and required implementation in relation to the year ended December 31, 2015. In accordance with the transitional regulations for the implementation of the CO the presentation of the prior-year financial statements was not adjusted to conform to the current presentation. Since Santhera prepares consolidated financial statements in accordance with International Financial Reporting Standards (IAASB), a recognized accounting standard, the Company has, in accordance with the CO, elected to forego presenting the statement of cash flows, the additional disclosures and the management report otherwise required by the CO.

#### Cash

Santhera holds cash balances, denominated mainly in Swiss francs (CHF) which include cash deposited in demand bank accounts, money market investment accounts and other liquid investments and interest earned on such cash balances.

#### Current assets and liabilities

Current assets are recorded at historical cost less adjustments for impairment of value and current liabilities at historical cost.

#### Loans to shareholdings

These are valued at their acquisition cost adjusted for impairment losses.

#### Investments in shareholdings

Investments in shareholdings are recorded at acquisition cost less adjustments for impairment of value. We evaluate our investments in subsidiaries for impairment annually and record an impairment loss when the carrying amount of such assets exceeds the fair value. We estimate fair value of our investments using a variety of valuation methods (e.g. income approach).

## Treasury shares

Treasury shares are recognized at acquisition cost and deducted from shareholders equity at the time of acquisition. In case of a resale, the gain or loss is recognized through the income statement as financial income or financial expenses.

## Related parties

In the meaning of the New Swiss Accounting Law, we consider related parties to be only shareholders, direct and indirect subsidiaries (shareholdings) and the board of directors.

## 3 Information on Balance Sheet and Income Statement Items

### 3.1 Loans to shareholdings

Loans to shareholdings are fully impaired to CHF 0 and consist of subordinated loans to Santhera Pharmaceuticals (Schweiz) AG. These loans were primarily related to fund the research and development activities of Santhera Group (December 31, 2015: CHF 172.4 million; December 31, 2014: CHF 172.4 million). The recoverability of these loans is not ensured. The fair value of Santhera Pharmaceuticals (Schweiz) AG and the long-term recoverability of these loans depend on the future market success of the developed and launched products (Raxone in LHON) and successful filings in other indications (Raxone in DMD).

### 3.2 Investments in shareholdings

In 2015 and 2014 the following companies are direct subsidiaries of Santhera Pharmaceuticals Holding AG (100% ownership and 100% voting rights):

	Share capital at December 31	2015	2014
Santhera Pharmaceuticals (Schweiz) AG Liestal, Switzerland	CHF	125,000	125,000
Santhera Pharmaceuticals (Deutschland) GmbH Lörrach, Germany	EUR	25,000	25,000
Santhera Pharmaceuticals (USA), Inc. Charlestown, US	USD	1,000	1,000
Santhera Pharmaceuticals (Canada), Inc. Montréal, Canada	CAD	1,000	1,000
Oy Santhera Pharmaceuticals (Finland) Ltd Helsinki, Finland	EUR	2,500	2,500

Santhera Pharmaceuticals (Schweiz) AG is the primary operational entity while Santhera Pharmaceuticals (Deutschland) GmbH holds the market authorization for the EU. Oy Santhera Pharmaceuticals (Finland) Ltd is not employing any personnel.

In 2015 the following companies, which are 100% direct subsidiaries (100% voting rights) of Santhera Pharmaceuticals (Schweiz) AG, were founded:

	Share capital at December 31	2015	2014
Santhera Pharmaceuticals (Liechtenstein) AG Ruggell, Fürstentum Liechtenstein	CHF	50,000	n/a
Santhera (Italy) S.r.l. Milano, Italy	EUR	50,000	n/a
Santhera (Germany) GmbH Munich, Germany	EUR	50,000	n/a
Santhera (Netherlands) B.V. Nieuwegein, The Netherlands	EUR	50,000	n/a
Santhera (UK) Limited London, United Kingdom	GBP	50,000	n/a

### 3.3 Share capital

During 2015, the share capital was increased by a total amount of CHF 1,288,306 to CHF 6,262,798 as of December 31, 2015 (2014: CHF 4,974,492): The increase consisted of three parts: i) increase through the exercise of 398,306 employee stock options (from conditional capital); ii) increase through an accelerated bookbuilding of 590,000 Shares (from authorized capital) and iii) the increase through the issuance of 300,000 Shares for the sale by an independent broker (from conditional capital).

### 3.4 Treasury shares

In connection with the liquidation of Oy Juvantia Pharma, Turku, Finland (**Juvantia**), acquired in 2009, Santhera received 8,028 Shares from former Juvantia shareholders. These treasury shares serve as pledge from the former owners of Juvantia for compensation of a potential tax claim related to pre-acquisition activities of Juvantia and were received in February 2010 at CHF 22 each. At December 31, 2015, the number of shares remained unchanged at 8,028.

### 3.5 Income from shareholdings

Income from shareholdings represents reimbursement for management services provided by the Company to its major shareholdings Santhera Pharmaceuticals (Schweiz) AG.

### 3.6 General and administrative expenses

	In CHF thousands	2015	2014
Administrative expenses		712	759
Consulting expenses		667	324
Expenses in connection with capital increases		1,943	380
<b>Total</b>		<b>3,322</b>	<b>1,463</b>

## 4 Other Information

### 4.1 Full-time equivalents

The number of full-time equivalents at period end was not above 10.

### 4.2 Significant shareholders (>2%)

Pursuant to information from the Company's share register and reporting of participations made to the Company in accordance with applicable stock exchange regulation, the following shareholders owned 2% or more of the Company's share capital as registered in the commercial register most recently at February 11, 2016 (6,262,798 shares at February 11, 2016; 4,578,521 shares at December 31, 2014):

	2015 Shares <sup>1</sup>	2015 %	2014 Shares	2014 %
Iglu Group, Switzerland	671,858	10.7	712,670	15.6
Consonance Capital Management, US	625,457	10.0	275,000	6.0
Bertarelli Ernesto, Donata and Maria-Iris, Switzerland	545,777	8.7	545,777	11.9
Union Asset Management Holding AG	326,838	5.2	n/a	n/a
Lagoda Investments Management, LLC, US	187,888	3.0	n/a	n/a
Visum Balanced Master Fund, Ltd., US	179,574	2.9	n/a	n/a
UBS Fund Management (Luxembourg) S.A.	167,203	2.7	n/a	n/a
RTW Investments, LTD, US	140,354	2.2	140,354	3.1
NGN Capital, Germany and US	n/a	n/a	137,409	3.0

<sup>1</sup> Including disclosures until March 30, 2016

<sup>2</sup> Formerly Ares Life Sciences, Switzerland

### 4.3 Disclosure of shares and stock options held by members of the Board and Executive Management (and their respective related party)

*As of December 31, 2015:*

	Number of Shares	Number of vested stock options	Number of unvested stock options	Total number of stock options
<i>Board of Directors</i>				
Martin Gertsch, Chairman	38,109	0	3,000	3,000
Jürg Ambühl	30,000	0	4,000	4,000
<i>Executive Management</i>				
Thomas Meier, CEO	72,902	0	12,250	12,250
Nicholas Coppard, SVP Head Development <sup>1</sup>	1	0	9,000	9,000
Günther Metz, SVP Business Development <sup>1</sup>	0	11,000	5,000	16,000
Christoph Rentsch, Chief Financial Officer <sup>2</sup>	0	0	15,000	15,000
Giovanni Stropoli, Chief Commercial Officer Europe and Rest of World <sup>1</sup>	400	0	15,000	15,000
Oliver Strub, SVP General Counsel and Secretary to the Board <sup>1</sup>	0	10,000	5,000	15,000

<sup>1</sup> Joined the Executive Management February 1, 2015.

<sup>1</sup> Joined the Executive Management July 1, 2015.

*As of December 31, 2014:*

	Number of Shares	Number of vested stock options	Number of unvested stock options	Total number of stock options
<i>Board of Directors</i>				
Martin Gertsch, Chairman	21,609	0	16,500	16,500
Jürg Ambühl	17,000	0	13,000	13,000
<i>Executive Management</i>				
Thomas Meier, CEO	38,508	48,644	59,500	108,144

#### 4.4. Disclosure of the allocation of stock options for Board of Directors, Executive Management and employees of Santhera Group

	2015	2015	2014	2014
	Quantity	Value (in TCHF) <sup>1</sup>	Quantity	Value (in TCHF) <sup>1</sup>
Board of Directors	7,000	282	29,500	116
Executive Management	53,500	2,094	52,000	93
Employees of Santhera Group	88,760	3,612	280,800	854
<b>Total</b>	<b>149,260</b>	<b>5,988</b>	<b>362,300</b>	<b>1,063</b>

<sup>1</sup> Value of the options calculated in accordance with the Hull-White model at the date of allocation in accordance with the terms of the award. The tax value of such stock options is 0 until stock options would be exercised. Such stock option values are theoretical values and do not reflect income tax values and do also take into consideration certain vesting provisions. For information about the underlying stock option plans, see note 17 "Stock Option Plans" in the consolidated financial statements. For information about the Company's compensation procedures, consult the Corporate Governance Report and the Compensation Report.

On January 1, 2016, 90,730 options were granted to employees of Santhera. These options are part of the bonus award for the year 2015 to employees of the Group. These options were granted under ESOP 2015 (see note 17 "Stock Option Plans").

	Quantity	Value (in TCHF) <sup>1</sup>
Executive Management	30,550	623
Employees of Santhera Group	60,180	1,226
<b>Total</b>	<b>90,730</b>	<b>1,849</b>

<sup>1</sup> Value of the options calculated in accordance with the Hull-White model at the date of allocation in accordance with the terms of the award. The tax value of such stock options is 0 until stock options would be exercised. Such stock option values are theoretical values and do not reflect income tax values and do also take into consideration certain vesting provisions. For information about the underlying stock option plans, see note 17 "Stock Option Plans" in the consolidated financial statements. For information about the Company's compensation procedures, consult the Corporate Governance Report and the Compensation Report.

#### 4.5 Contingencies and guarantees

##### *Guarantee towards Swiss VAT authorities*

The Company is part of the value-added tax group of the Swiss affiliated companies of Santhera Pharmaceuticals and is therefore jointly and severally liable to the Swiss federal tax administration for their value-added tax liabilities.

##### *Guarantee towards Santhera Pharmaceuticals (Schweiz) AG*

The Company guarantees to pay for the liabilities of its subsidiary Santhera Pharmaceuticals (Schweiz) AG until the Annual Shareholders' Meeting in 2016.

##### *Declaration of liability towards Arval Deutschland GmbH*

The Company guarantees to pay for the liabilities of its subsidiary Santhera (Germany) GmbH for contractual duties and obligations.

#### **4.6 Standby Equity Distribution Agreement**

In October 2013, Santhera announced that it has entered into a Standby Equity Distribution Agreement (**SEDA**) with Yorkville Advisors Global Master SPV Ltd., New York, US (**YA Global**). Under the terms of the agreement, YA Global has committed to provide up to CHF 10 million in equity financing during a period of three years. The SEDA has been established in order to support the funding of Santhera's operations. It remains at the sole discretion of Santhera to determine the timing of the funding. The remaining amount available for equity financing with YA Global, sums up to CHF 8.1 million.

#### **4.7 Events After the Reporting Date**

None

## Report of the Statutory Auditor on the Financial Statements

Basel, April 11, 2016

As statutory auditor, we have audited the financial statements of Santhera Pharmaceuticals Holding AG, which comprise the balance sheet, income statement and notes (pages 53 to 61), for the year ended 31 December 2015.

### **Board of Directors' responsibility**

The Board of Directors is responsible for the preparation of the financial statements in accordance with the requirements of Swiss law and the company's articles of incorporation. This responsibility includes designing, implementing and maintaining an internal control system relevant to the preparation of financial statements that are free from material misstatement, whether due to fraud or error. The Board of Directors is further responsible for selecting and applying appropriate accounting policies and making accounting estimates that are reasonable in the circumstances.

### **Auditor's responsibility**

Our responsibility is to express an opinion on these financial statements based on our audit. We conducted our audit in accordance with Swiss law and Swiss Auditing Standards. Those standards require that we plan and perform the audit to obtain reasonable assurance whether the financial statements are free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the financial statements. The procedures selected depend on the auditor's judgment, including the assessment of the risks of material misstatement of the financial statements, whether due to fraud or error. In making those risk assessments, the auditor considers the internal control system relevant to the entity's preparation of the financial statements in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the entity's internal control system. An audit also includes evaluating the appropriateness of the accounting policies used and the reasonableness of accounting estimates made, as well as evaluating the overall presentation of the financial statements. We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our audit opinion.

### **Opinion**

In our opinion, the financial statements for the year ended 31 December 2015 comply with Swiss law and the company's articles of incorporation.

**Report on other legal requirements**

We confirm that we meet the legal requirements on licensing according to the Auditor Oversight Act (AOA) and independence (article 728 CO and article 11 AOA) and that there are no circumstances incompatible with our independence.

In accordance with article 728a paragraph 1 item 3 CO and Swiss Auditing Standard 890, we confirm that an internal control system exists, which has been designed for the preparation of financial statements according to the instructions of the Board of Directors.

We recommend that the financial statements submitted to you be approved.

Ernst & Young Ltd

/s/ Jolanda Dolente  
Licensed audit expert  
(Auditor in charge)

/s/ Nicole Riggerbach  
Licensed audit expert

(This page has been left blank intentionally.)

**THE COMPANY**

**Santhera Pharmaceuticals Holding AG**

Hohenrainstrasse 24  
4133 Pratteln  
Switzerland

**LEGAL ADVISORS TO THE COMPANY**

*As to Swiss law*

**Homburger AG**

Prime Tower  
Hardstrasse 201  
8005 Zurich  
Switzerland

*As to U.S. law*

**Allen & Overy LLP**

Haus am OpernTurm  
Bockenheimer Landstrasse 2  
60306 Frankfurt am Main  
Germany

**LEGAL ADVISORS TO THE MANAGERS**

*As to Swiss law*

**Bär & Karrer AG**

Brandschenkestrasse 90  
8027 Zurich  
Switzerland

*As to U.S. law*

**Linklaters LLP**

Taunusanlage 8  
60329 Frankfurt am Main  
Germany

**INDEPENDENT AUDITORS OF THE COMPANY**

**Ernst & Young AG**

Aeschengraben 9  
4051 Basel  
Switzerland