santhera Pharmaceuticals

2015

Interim Report
January to June 2015

Report on the Six Months Ending June 30, 2015, and Interim Consolidated Financial Statements

Content

Santhera Reports Transitions Towards Product Company

Santhera Pharmaceuticals transformed into a specialty pharmaceutical company with sustainable revenues from product sales. In June 2015, the Committee for Medicinal Products for Human Use (CHMP) recommended granting a marketing authorization in Europe for Raxone® for the treatment of Leber's Hereditary Optic Neuropathy (LHON) and Santhera expects a final decision from the European Commission (EC) very soon. In parallel, the Company advances Raxone®/Catena® with high priority towards approval in Duchenne Muscular Dystrophy (DMD) in Europe and the USA.

Key financials in the First Half Year 2015:

- **Preparation for product launch increased cash burn during six-month period**

  As of June 30, 2015, Santhera had cash and cash equivalents of CHF 10.5 million. Net change in cash and cash equivalents in the first half year of 2015 increased to CHF –7.0 million (1H 2014: CHF 0 million) as a result of the ongoing preparation to launch Raxone® in Europe.

- **Increase in sales of Raxone®**

  In the first six months of 2015, Raxone® generated net sales of CHF 1.5 million (1H 2014: CHF 0.8 million), driven by increased product sales for LHON patients under the temporary authorization cATU in France as well as sales under named patient and special access programs.

- **Higher operating expenses due to launch preparations and expanded operations**

  Operating expenses of CHF 7.4 million (1H 2014: CHF 3.9 million) were comprised of CHF 2.8 million in development, CHF 1.4 million in marketing and sales expenses, and CHF 3.1 million in general and administrative expenses (G&A). The increase reflects the hiring of a commercial team for the product launch, higher expenses in development due to preparation of regulatory filings and higher expenses in G&A resulting from the expansion of Santhera's operations. Consequently, the operating loss was CHF –6.0 million (1H 2014: CHF –3.1 million) leading to a net result of CHF –6.2 million (1H 2014: CHF –3.1 million).

Product and Pipeline Highlights:

- **Received positive opinion from the CHMP**

  In June 2015, the CHMP recommended granting a marketing authorization for Raxone® for the treatment of visual impairment in adolescent and adult patients with LHON. The formal marketing authorization from the EC for the product is expected shortly.

- **Advanced preparation for product launch**

  In recent months, the Company prepared for a fast and efficient launch of Raxone® which is planned for the first countries in coming weeks subject to EC approval.

- **Published outcome of Phase III study in *The Lancet* and prepares for regulatory filings for Raxone®/Catena® in DMD in Europe and the USA**

  Earlier this year, the positive outcome of the Phase III study was published in The Lancet and presented at the annual meeting of the American Academy of Neurology. Based on available data from Santhera's development program with Raxone®/Catena® in DMD and supplemented by natural history data which we obtain from a collaboration with the Cooperative International Neuromuscular Research Group (CINRG), Santhera plans to submit a New Drug Application (NDA) in the USA and Marketing Authorization Application (MAA) as a variation to the EC approval in LHON. In April 2015, the US FDA has granted Fast Track Designation for Raxone®/Catena® for the treatment of DMD.

- **Started dosing of first Congenital Muscular Dystrophy (CMD) patient with omigapil and completed patient recruitment in collaborative study with US National Institutes of Health (NIH)**

  The first patient in the CALLISTO Phase I trial was dosed with oral omigapil in a new formulation developed by Santhera for use in children. At the same time, all 20 participants required for the study have been selected, prescreened and randomly assigned to one of the three study cohorts. The study is being conducted at the NIH's National Institute of Neurological Disorders and Stroke in Bethesda (Maryland, USA) and assesses the pharmacokinetics, safety, and tolerability of omigapil in ambulatory and non-ambulatory children affected by either Ullrich or MDC1A subtypes of CMD.

Corporate Highlights:

- **Expanded Executive Management and started building a commercial team**

  In the first half year of 2015, Santhera nominated three senior members of staff to its Executive Management and newly appointed a Chief Commercial Officer and a Chief Financial Officer in a step to prepare the Company for its growth strategy. Simultaneously, the Company started building a commercial team with experience in successfully managing the international commercialization of orphan and niche-market drug products.

Outlook:

In Europe, after approval from the EC, Raxone® will be commercially launched in the first countries for the treatment of LHON starting in the second half of 2015. The Company continues its interactions with regulatory authorities and plans to submit NDA and MAA filings for DMD based on the positive Phase III DELOS trial, the natural history study from the CINRG, and other supportive evidence of efficacy. The Company believes that, with CHF 37.0 million as of end of August 2015, it has sufficient cash to support the commercial introduction of Raxone® in Europe and the currently planned development and regulatory programs.

Interim Consolidated Financial Statements

Content

Interim Consolidated Balance Sheet

in CHF thousands	Notes	June 30, 2015 (reviewed)	Dec. 31, 2014 (audited)
Assets			
Tangible assets		171	132
Intangible assets		3,762	4,197
Financial assets long-term		85	85
Noncurrent assets	9	**4,018**	**4,414**
Prepaid expenses and accrued income		345	376
Inventories	6	2,787	0
Trade and other receivables		1,285	720
Receivables from shareholders		145	0
Cash and cash equivalents	7	10,476	17,435
Current assets		**15,038**	**18,531**
Total assets		**19,056**	**22,945**
Equity and liabilities			
Share capital	8	5,049	4,974
Capital reserves and share premium		294,424	293,232
Retained earnings		−279,048	−272,838
Employee benefit reserve		−1,193	−1,287
Treasury shares		−177	−177
Other components of equity (translation differences)		−7,171	−6,666
Total equity		**11,884**	**17,238**
Pension liabilities		2,696	2,680
Total noncurrent liabilities		**2,696**	**2,680**
Trade and other payables		2,800	2,166
Accrued expenses		1,676	861
Total current liabilities		**4,476**	**3,027**
Total liabilities		**7,172**	**5,707**
Total equity and liabilities		**19,056**	**22,945**

Interim Consolidated Income Statement (Reviewed)

for the half year ended June 30, in CHF thousands	Notes	2015	2014
Net sales	9	1,455	829
Cost of goods sold		−159	−75
Gross profit		**1,296**	**754**
Other operating income		23	23
Development	10	−2,846	−1,913
Marketing and sales	10	−1,415	−157
General and administrative	10	−3,101	−1,790
Other operating expenses	10	−4	0
Operating expenses	**10**	**−7,366**	**−3,860**
Operating result		**−6,047**	**−3,083**
Financial income		104	10
Financial expenses		−266	−20
Result before taxes		**−6,209**	**−3,093**
Income taxes		−2	−1
Net result		**−6,211**	**−3,094**
Basic and diluted loss per share (in CHF)		**−1.24**	**−0.70**

Interim Consolidated Statement of Comprehensive Income (Reviewed)

for the half year ended June 30, in CHF thousands	2015	2014
Net result	**−6,211**	**−3,094**
Items never to be reclassified subsequently to net income in subsequent periods:		
Actuarial gains/(losses) on defined benefit plans	94	−322
Items to be reclassified subsequently to net income in subsequent periods:		
Currency translation differences	−505	−29
Other comprehensive result	**−411**	**−351**
Total comprehensive result	**−6,622**	**−3,445**

Interim Consolidated Statement of Cash Flows (Reviewed)

for the half year ended June 30, in CHF thousands	Notes	2015	2014
Result before taxes		−6,209	−3,093
Depreciation of tangible assets		33	35
Amortization and impairment of intangible assets		7	4
Expenses for share options		604	338
Change in pension liabilities		110	46
Taxes paid		−2	−1
Change in net working capital		−1,873	93
Total financial result		162	10
Interest received		1	0
Interest paid		0	−4
Cash flow from operating activities		**−7,167**	**−2,572**
Investments in tangible assets		−73	−149
Investments in intangible assets		−59	−17
Cash flow from investing activities		**−132**	**−166**
Proceeds from private placement	8	0	1,000
Proceeds from option exercise	8	519	316
Proceeds from sale of treasury shares SEDA[1]	8	0	1,444
Cost of issuance share capital		0	−22
Cash flow from financing activities		**519**	**2,738**
Effects of exchange rate changes on cash and cash equivalents		−179	−4
Net increase/(decrease) in cash and cash equivalents		**−6,959**	**−4**
Cash and cash equivalents at January 1		17,435	5,044
Cash and cash equivalents at June 30		**10,476**	**5,040**

[1] Standby Equity Distribution Agreement (see note 8 *"Share Capital"*)

Interim Consolidated Statement of Changes in Equity (Reviewed)

in CHF thousands	Notes	Share capital	Capital reserves and share premium	Retained earnings	Employee benefit reserve	Treasury shares	Translation differences	Total
Balance at January 1, 2014		**3,934**	**274,896**	**−265,304**	**405**	**−221**	**−6,604**	**7,106**
Net result		0	0	−3,094	0	0	0	−3,094
Other comprehensive income		0	0	0	−322	0	−29	−351
Total comprehensive result for the period		**0**	**0**	**−3,094**	**−322**	**0**	**−29**	**−3,445**
Share-based payment transactions	10	0	338	0	0	0	0	338
Capital increase option exercise		92	224	0	0	0	0	316
Capital increase SEDA[1]		355	1,045	0	0	44	0	1,444
Capital increase private placement		288	712	0	0	0	0	1,000
Cost of issuance share capital		0	−22	0	0	0	0	−22
Balance at June 30, 2014		**4,669**	**277,193**	**−268,398**	**83**	**−177**	**−6,633**	**6,737**
Balance at January 1, 2015		**4,974**	**293,232**	**−272,838**	**-1,287**	**−177**	**−6,666**	**17,238**
Net result		0	0	−6,211	0	0	0	−6,211
Other comprehensive income		0	0	0	94	0	−505	−411
Total comprehensive result for the period		**0**	**0**	**−6,211**	**94**	**0**	**−505**	**−6,622**
Share-based payment transactions	10	0	604	0	0	0	0	604
Capital increase option exercise		75	589	0	0	0	0	664
Balance at June 30, 2015		**5,049**	**294,425**	**−279,049**	**−1,193**	**−177**	**−7,171**	**11,884**

[1] Standby Equity Distribution Agreement (see note 8 *"Share Capital"*)

Notes to the Unaudited 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 neuromuscular and mitochondrial diseases, an area which includes many orphan and niche indications with no current therapy.

The Company, having the listing of its registered shares (**Shares**) on the SIX Swiss Exchange, is a Swiss stock corporation and the parent company of the Group. The Company has its registered offices at Hammerstrasse 49 in 4410 Liestal, Switzerland.

The consolidated interim financial statements were approved for publication by the Board of Directors (**Board**) on September 2, 2015.

2 Business Update

Following the Marketing Authorization Application (**MAA**) with the European Medicines Agency in Leber's Hereditary Optic Neuropathy (**LHON**) in 2014, the Committee for Medicinal Products for Human Use (**CHMP**) has recommended granting a marketing authorization for Raxone® for the treatment of LHON end of June 2015.

Through the gross cash-in of CHF 27.7 million received in August 2015 (see note 13 *"Subsequent Events"*), and product sales under the temporary approval in France (Autorisation temporaire d'utilisation dite de cohorte – **cATU**) for Raxone® in the treatment of LHON and international Named Patient Programs (**NPP**) the Company has sufficient cash to support its currently planned development and regulatory programs and to make necessary preparations for the commercial introduction of Raxone® in Europe.

The Company expects to receive the marketing authorization from the European Commission (**EC**) in the third quarter of 2015. Upon receipt of such authorization, Santhera will launch the product in the European Union (**EU**) and generate income from product sales in the first countries.

The positive outcome of a phase III trial in Duchenne Muscular Dystrophy (**DMD**) in May 2014 forms the basis for regulatory filings in the EU and the United States of America (**USA**). Depending on the potential regulatory filing in DMD and the related commercialization strategy of Raxone®/Catena® particularly in the USA, Santhera may require additional funds in order to finance its activities until revenues reach a level to sustain positive cash flows.

3 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, 2014, except for the adoption of new standards and interpretations as of January 1, 2015, as noted below.

Basis of preparation

These unaudited consolidated interim financial statements were prepared in accordance with IAS 34 Interim Financial Reporting and should be read in conjunction with the annual financial statements for the year ended December 31, 2014.

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.

Changes in accounting policies

The accounting policies adopted in the preparation of the interim condensed 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, 2014.

The following new or revised standards were adopted but did not have any material impact or lead to additional disclosures but may affect the accounting for future transactions or arrangements:

- Annual Improvements Cycle – 2010–2012
- Annual Improvements Cycle – 2011–2013
- Defined Benefit Plans: Employee Contributions (Amendments to IAS 19)

4 Seasonality

The operating result is not subject to significant seasonal variations during the financial year.

5 Exchange Rates of Principal Currencies

	Income statement in CHF average rates		**Balance sheet in CHF** as of period end	
	Six months ended June 30, 2015	Six months ended June 30, 2014	**June 30, 2015**	Dec. 31, 2014
1 euro (**EUR**)	**1.0583**	1.2213	**1.0365**	1.2028
1 US dollar (**USD**)	**0.9475**	0.8909	**0.9342**	0.9895
1 Canadian dollar (**CAD**)	**0.7680**	0.8126	**0.7561**	0.8510

6 Inventories

This position consists mainly of an active pharmaceutical ingredient which is kept by Santhera as stock for launch and inventory risk management purposes (security stock) for Raxone®/Catena®. Due to increased sales and sales forecast the Company reversed prior period impairment in the amount of CHF 1.1 million, recognized in the operating expenses under "Development".

7 Cash and Cash Equivalents

in CHF thousands	**June 30, 2015**	Dec. 31, 2014
Cash at banks and on hand		
in CHF	9,937	16,416
in EUR	444	724
in USD	41	267
in CAD	54	28
Total at period end	**10,476**	**17,435**

8 Share Capital

Ordinary share capital

During the reporting period ending June 30, 2015, 74,721 Shares were issued out of conditional share capital upon the exercise of stock options. As a result, as of June 30, 2015, the issued nominal share capital amounted to CHF 5,049,213, divided into 5,049,213 Shares at a nominal value of CHF 1 each.

In the same period for 2014, 91,634 Shares were issued from conditional capital upon the exercise of stock options Santhera issued 355,000 additional Shares from conditional capital under the Standby Equity Distribution Agreement. In February 2014, additional 288,317 Shares were issued from authorized capital for a private placement.

Authorized share capital

On the occasion of the Annual Shareholders' Meeting (**ASM**) on May 11, 2015, Santhera's 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.

Conditional share capital

As of June 30, 2015, the Company had conditional share capital, pursuant to which the share capital may be increased by

(i) a maximum amount of CHF 800,000 through the issuance of up to 800,000 Shares with the exercise of option rights. This part of the conditional share capital was increased from formerly CHF 604,029, as per December 31, 2014, to CHF 800,000 as approved at the ASM on May 11, 2015. During the first half of 2015, 74,721 options were exercised, reducing the available conditional capital to CHF 725,279 as per June 30, 2015. In the same period 2014, 91,634 options were exercised, reducing the available conditional capital to CHF 709,521 as per June 30, 2014 (see note 11 *"Stock Option Plans")*.

(ii) a maximum amount of CHF 950,000 by issuing up to 950,000 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. This part of the conditional share capital was increased from formerly CHF 600,000, as per December 31, 2014, to CHF 950,000 as approved at the ASM on May 11, 2015.

9 Segment and Geographic Information

Segment information

Santhera operates in one business segment, namely development and commercialization of products for the treatment of neuromuscular and mitochondrial diseases. The Board and the Executive Management, 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 under the cATU in France of Raxone® for LHON and of Raxone® and Catena® under NPP. Geographic revenue information is based on location of the customer.

Geographic information

Net sales

six months ended June 30, in CHF thousands	**2015**	2014
Europe	1,455	829
Total	**1,455**	**829**

Noncurrent assets (excluding financial instruments and deferred tax assets)

in CHF thousands	**June 30, 2015**	Dec. 31, 2014
EU	3,036	3,523
Switzerland	895	803
North America	2	3
Total	**3,933**	**4,329**

10 Operating Expenses by Nature

six months ended June 30, in CHF thousands	**2015**	2014
External development expenses	−2,290	−777
Patent and license expenses	−98	−103
Marketing expenses	−320	−83
Employee expenses	−4,404	−1,964
of which non-cash-relevant expenses for share-based payments	*−604*	*−338*
General and administrative expenses	−1,160	−761
Depreciation, amortization and impairment	−40	−39
Reversal of impairment on inventories	1,111	0
Lease expenses	−161	−133
Other operating expenses	−4	0
Total operating expenses	**−7,366**	**−3,860**

11 Stock Option Plans

Santhera has established Employee Stock Option Plans (**ESOP**), the ESOP 2004, the ESOP 2008, the ESOP 2010, the ESOP 2015, the 2006 Executive Incentive Plan (**EIP**) and Board Stock Option Plans (**BSOP**), the BSOP 2011 and BSOP 2015 to align the long-term interests of the Board, the Executive Management, employees and consultants. Options granted under these stock option plans are equity-settled. New grants are only possible currently under the ESOP 2015 and BSOP 2015.

In the reporting period ended June 30, 2015, a total of 94,260 options with exercise prices between CHF 84.10 and CHF 105.50 were granted. In the half-year period ending June 30, 2014, a total of 352,000 options with exercise prices between CHF 3.78 and CHF 4.02 were granted.

The fair value of stock options is determined at each grant date by using the Hull-White option pricing model. For the calculation of the fair value of stock options granted during the reporting period in 2015, the same range of valuation parameters as disclosed in the financial statements as of December 31, 2014, was applied, except for the CHF risk-free interest rate (between 0.00% and 0.38%). The non-cash-relevant expenses for all unvested stock options in the reporting period 2015 amounts to TCHF 604 compared to TCHF 338 in the same period in 2014.

Options outstanding

six months ended June 30, number of options	**2015**	2014
At January 1	**477,580**	**323,421**
Granted[1]	94,260	352,000
Forfeited	−2,700	0
Expired	0	−5,215
Exercised	−74,721	−91,634
At June 30[2]	**494,419**	**578,572**

[1] The weighted average fair value of the stock options granted during the reporting period in 2015 was CHF 43.09 (CHF 1.96 in the comparative reporting period 2014).

[2] Based on the closing price of CHF 90.00 of the Santhera Shares on June 30, 2015, a total of 440,166 stock options were in the money, whereof 381,431 were vested (on June 30, 2014, the closing Share price was CHF 46.95; a total of 197,022 options were in the money, whereof 117,207 were vested).

12 Related Party Transactions

During the reporting period 2015, a total of 7,000 options were granted to members of the Board and 53,500 options were granted to members of the Executive Management. In the same period in 2014, a total of 28,000 options were granted to members of the Board and 52,000 options to the then sole member of the Executive Management.

13 Subsequent Events

On August 7, 2015, Santhera announced the completion of the sale of 300,000 registered shares of common stock at an average price of CHF 92.38 each. The Company received CHF 27.7 million in gross proceeds from the sale. The Shares were sold by the independent broker Kepler Cheuvreux within a period of four days. The treasury shares with a par value of CHF 1.00 were issued from the Company's conditional capital.

Report on the Review of Interim Condensed Consolidated Financial Statements

Basel, 2 September 2015

Introduction

We have reviewed the interim condensed consolidated financial statements (consolidated balance sheet, consolidated income statement, consolidated statement of comprehensive income, consolidated statement of cash flows, consolidated statement of changes in equity and explanatory notes) of Santhera Pharmaceuticals Holding AG for the six-month period ended 30 June 2015 (pages 5 to 14). The Board of Directors is responsible for the preparation and presentation of these interim condensed consolidated financial statements in accordance with IAS 34 "Interim Financial Reporting". Our responsibility is to express a conclusion on these interim condensed consolidated financial statements based on our review.

Scope of Review

We conducted our review in accordance with International Standard on Review Engagements 2410 "Review of Interim Financial Information Performed by the Independent Auditor of the Entity". A review of interim financial information consists of making inquiries, primarily of persons responsible for financial and accounting matters, and applying analytical and other review procedures. A review is substantially less in scope than an audit conducted in accordance with International Standards on Auditing and consequently does not enable us to obtain assurance that we would become aware of all significant matters that might be identified in an audit. Accordingly, we do not express an audit opinion.

Conclusion

Based on our review, nothing has come to our attention that causes us to believe that the interim condensed consolidated financial statements are not prepared, in all material respects, in accordance with International Financial Reporting Standard IAS 34 "Interim Financial Reporting".

Ernst & Young AG

Jolanda Dolente
Licensed audit expert
(Auditor in charge)

Nicole Riggenbach
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Forward-Looking Statements

This Interim Report expressly or implicitly contains certain forward-looking statements concerning Santhera Pharmaceuticals Holding AG and its business. Such statements involve certain known and unknown risks, uncertainties and other factors, which could cause the actual results, financial condition, performance or achievements of Santhera Pharmaceuticals Holding AG to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. There can be no guarantee that any of the development projects described will succeed or that any new products or indications will be brought to market. Similarly, there can be no guarantee that Santhera Pharmaceuticals Holding AG or any future product or indication will achieve any particular level of revenue. In particular, management's expectations could be affected by, among other things, uncertainties involved in the development of new pharmaceutical products, including unexpected clinical trial results; unexpected regulatory actions or delays or government regulation generally; the Company's ability to obtain or maintain patent or other proprietary intellectual property protection; competition in general; government, industry, and general public pricing and other political pressures. Santhera Pharmaceuticals Holding AG is providing the information in this Interim Report as of the date of the publication, and does not undertake any obligation to update any forward-looking statements contained herein as a result of new information, future events or otherwise.

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