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A conference call will be held today at 13:00 CET, 12:00 GMT, 08:00 EDT. Details are at the end of this statement.

Santhera Reports 2019 Annual Results and Highlights Pipeline Progress in Duchenne Muscular Dystrophy

- **Net revenues from product sales of CHF 27.9 million, slightly above guidance**
- **Net revenue of CHF 46.4 million recognized from out-licensing agreement with Chiesi Group for Raxone®**
- **Markedly improved operating result of CHF -10.4 million (2018: CHF -51.4 million) and net result of CHF -19.0 million (2018: CHF -54.2 million), largely owing to out-licensing income**
- **Cash and cash equivalents of CHF 31.4 million as of December 31, 2019**
- **Strong progress in advancing lead Duchenne muscular dystrophy (DMD) compounds Puldysa® (idebenone) and vamorolone towards market entry and regulatory submission, respectively.**
- **Board of Directors to propose increase of authorized and conditional capital at upcoming Annual General Meeting**

Pratteln, Switzerland, March 24, 2020 – Santhera Pharmaceuticals (SIX: SANN) announces the Company's audited financial results for 2019 and reports on progress in advancing its lead compounds for the treatment of Duchenne muscular dystrophy (DMD), Puldysa® (idebenone) and vamorolone, towards market entry and regulatory submission, respectively.

"Santhera is emerging as a leader in addressing rare neuromuscular diseases and 2019 saw us prepare the business for several significant value inflection points this year for our drug candidates to treat DMD," said **Dario Eklund, CEO of Santhera**. "The regulatory review for Puldysa in the EU, where we are seeking a conditional marketing authorization (CMA) for the treatment of respiratory dysfunction in patients with DMD who are not using glucocorticoids, is on track and we anticipate obtaining an opinion from the European regulators in mid-2020. Our goal is to provide the first approved drug in non-ambulant DMD patients who are in respiratory decline and we are planning for launch in the first European markets by the end of the year.

"Our second DMD product, vamorolone, is currently being investigated by the originator company ReveraGen in a pivotal study (called VISION-DMD) in ambulant patients with DMD, and our ambition is to replace glucocorticoids as standard of care. We expect read-out of topline data from this trial in the fourth quarter 2020, followed by filing of a US new drug application (NDA) in the first quarter of 2021. Positive data will also pave the way for Santhera to exercise its sub-licensing option for the product."

2019 full-year net revenues slightly above guidance

In 2019, Santhera reported net revenues from product sales of CHF 27.9 million (2018: CHF 31.7 million), slightly surpassing the Company's full-year guidance. This includes sales of Raxone for the treatment of Leber's hereditary optic neuropathy (LHON) in Europe in the first seven months of 2019 and in France for the full year.

Upfront payment from Chiesi Group following closing of licensing agreement

In August 2019, Santhera received an upfront payment of CHF 49.3 million (EUR 44 million) in connection with entering into the licensing agreement with Chiesi Group. The majority of this payment (CHF 46.4 million) was recognized as revenue in 2019 and the remainder will be accounted for in line with the completion of some related obligations. As previously announced per the agreement, Chiesi Group has in-licensed Raxone for LHON and all other ophthalmologic indications for all territories worldwide except the US and Canada for a total consideration of up to EUR 93 million.

Operating and net result markedly improved, largely owing to licensing income

With CHF 80.7 million, total operating expenses slightly surpassed the previous year's level (2018: CHF 78.7 million). The increase in development expenses to CHF 41.2 million (2018: CHF 38.2 million) reflects expenses for ongoing late stage clinical studies such as the Phase 3 SIDEROS trial in DMD, efforts associated with the pending marketing authorization application for Puldysa for DMD in Europe, ongoing post-authorization studies for LHON, as well as clinical work with Santhera's early stage development compound POL6014 for cystic fibrosis. Cost savings were achieved for marketing and sales, which amounted to CHF 20.1 million (2018: CHF 24.9 million), as commercial activities were aligned with market entry for Puldysa expected towards end of 2020. General and administrative expenses increased to CHF 19.2 million (2018: CHF 15.4 million) due to one-time expenses related to the transaction with Chiesi Group. Largely owing to the first installment from Chiesi Group in the context of the licensing agreement, the Company reported a markedly improved operating result of CHF -10.4 million (2018: CHF -51.4 million). For the full-year 2019, Santhera reported a net result of CHF -19.0 million (2018: CHF -54.2 million).

Growth plans necessitate additional funds

As of December 31, 2019, freely available liquid funds (cash and cash equivalents) amounted to CHF 31.4 million (August 31, 2019: CHF 43.7 million). In addition, the Company held CHF 1.5 million of restricted cash designated for the interest payments related to the convertible bonds issued in 2017.

Ongoing development activities, ramping up of the commercialization activities relating to Puldysa® and the intention to exercise the option to obtain an exclusive sub-license from Idorsia to commercialize ReveraGen's vamorolone will require substantial additional funding, particularly in the latter part of 2020. Material uncertainties thus remain as to the Company's ability to continue as a going concern until December 31, 2020. Executing the Company's strategy depends on further funding to ensure the continuation of its operations through December 31, 2020.

Santhera is currently evaluating a number of different options to secure additional financing of the Company which besides equity-based funding also includes debt financing, royalty financing, standby equity distribution agreement as well as the monetization of receivables.

At the forthcoming Annual General Meeting on April 22, 2020, the Board will propose the increase of authorized capital from CHF 3,000,000 to CHF 5,500,000 and an increase of conditional capital from CHF 2,500,000 to CHF 4,800,000.

Subject to approval by the Company's shareholders, this would enable Santhera to raise new capital later in 2020 to support the achievement of regulatory and commercial milestones for Puldysa and vamorolone, to exercise the license option for vamorolone and to further strengthen its resources, especially in view of important product launches planned for 2020/2021. Furthermore, the Company is currently evaluating a restructuring of the CHF 60 million Senior Unsecured Convertible Bonds due in February 2022 including a significant reduction of the conversion price which would increase the likelihood of conversion into shares rather than repayment.

Pipeline projects on track to reach key near-term inflection points

Anticipated near-term inflection points towards approval for both DMD pipeline candidates Puldysa (idebenone) and vamorolone are:

- mid-2020: CHMP opinion on marketing authorization application for Puldysa in DMD in Europe
- Q4-2020: Launch of Puldysa in first European markets
- Q4-2020: Read-out of topline data of pivotal trial for vamorolone in DMD
- Q1-2021: Filing a New Drug Application for vamorolone in DMD in the US

Santhera's strategic priorities for 2020 are its neuromuscular franchise: Puldysa and vamorolone in DMD. For Puldysa, the focus is on the preparation for European market entry in DMD later in the year and the completion of enrollment into the SIDEROS trial to support planned regulatory submissions, particularly in the US. For vamorolone, the key milestone will be the VISION-DMD topline 6-month data readout, which if positive would allow for preparation of the NDA filing and pave the way for Santhera's option exercise.

In parallel, the Company is advancing its clinical stage candidate POL6014 for cystic fibrosis and is evaluating further diversification of its platform type pipeline products, including development of additional indications in collaboration with partners. The pipeline also includes a discovery-stage gene therapy approach targeting congenital muscular dystrophies.

Further information about Santhera's development pipeline is available in the **Annual Report 2019** which can be viewed here.

Statement on the impact of the COVID-19 outbreak

Santhera's first priority remains the health and safety of its employees and clinical study participants. As a priority, the Company is closely monitoring the impact of COVID-19 on its operations to continue meeting the needs of the patients and securing an uninterrupted supply of medication to both clinical trials participants and patients enrolled in expanded access programs.

Santhera continues to expect an opinion from the Committee for Medicinal Products for Human Use (CHMP) on its marketing authorization application for Puldysa by mid-2020. As the European Medicines Agency already announced conducting virtual meetings, the Company does not expect a delay related to COVID-19.

Annual Report

The Santhera Annual Report 2019 is available for download on the Company's website at www.santhera.com/investors-and-media/investor-toolbox/financial-reports.

Annual General Meeting 2020

On March 16, 2020, the Swiss Federal Council, in response to the situation regarding the coronavirus SARS-Cov-2, has prohibited all public and private events in Switzerland until April 19, 2020, and permitted companies to provide that their shareholders exercise their rights solely by way of giving voting instructions to the independent proxy. On this basis, the Company hereby mandates that all shareholders exercise their rights at the Annual General Meeting (AGM) of April 22, 2020, solely via the independent proxy. There is no possibility to attend the AGM in person.

The invitation which will be sent to registered shareholders can be downloaded from www.santhera.com/investors-and-media/investor-toolbox/shareholder-meetings and information on how to issue power of attorney and instructions to the independent proxy, electronically or in writing, can be found on page 10 of this invitation.

Conference Call

Santhera will host a conference call on March 24, 2020 at 13:00 CET / 12:00 GMT / 07:00 EDT. Dario Eklund, CEO of Santhera, will discuss the 2019 financial results and will provide an update on corporate developments. Participants are invited to call one of the following numbers 10-15 minutes before the conference call starts (no dial-in code is required):

Europe: +41 58 310 50 00
UK: +44 207 107 06 13
USA: +1 631 570 56 13

2019 Full-year Financial Information

Santhera's 2019 Annual Report see www.santhera.com/investors-and-media/investor-toolbox/financial-reports.

Condensed consolidated income statement (IFRS, in CHF thousands)	**2019**	2018
Net sales	**27,890**	31,657
Revenue from out-licensing transactions	**46,370**	0
Net sales to licensing partner	**1,116**	0
Revenue from contracts with customers	**75,376**	31,657
Cost of goods sold (of which amortization intangible assets: 2019 -3,039 / 2018 -3,039)	**-5,450**	-4,702
Development	**-41,244**	-38,240
Marketing and sales	**-20,096**	-24,884
General and administrative	**-19,184**	-15,365
Operating expenses	**-80,652**	-78,687
Operating result	**-10,442**	-51,420
Financial result	**-7,952**	-2,444
Income taxes	**-579**	-322
Net result	**-18,973**	-54,186
Basic and diluted loss per share (in CHF)	**-1.73**	-7.86

Condensed consolidated balance sheet (IFRS, in CHF thousands)	**2019**	2018
Cash and cash equivalents	**31,358**	21,971
Other current assets	**17,897**	21,112
Noncurrent assets	**65,796**	67,211
Total assets	**115,051**	110,294
Equity	**21,247**	27,829
Noncurrent liabilities	**69,840**	62,756
Current liabilities	**23,964**	19,709
Total equity and liabilities	**115,051**	110,294

Condensed consolidated cash flow statement (IFRS, in CHF thousands)	**2019**	2018
Operating cash flow	**2,595**	-37,865
Investing cash flow	**2,789**	-5,909
Financing cash flow	**4,129**	20,644
Cash and cash equivalents at January 1	**21,971**	45,195
Cash and cash equivalents at December 31	**31,358**	21,971
Net change in cash and cash equivalents	**9,387**	-23,224

Share capital (number of shares with par value of CHF 1)	**2019**	2018
Shares issued	**11,165,063**	10,664,563
Conditional capital for equity rights	**687,052**	687,552
Conditional capital for convertible rights	**2,500,000**	930,000
Authorized capital	**3,000,000**	500,000

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare neuromuscular and pulmonary diseases with high unmet medical need. Santhera is building a Duchenne muscular dystrophy (DMD) product portfolio to treat patients irrespective of causative mutations, disease stage or age. A marketing authorization application for Puldysa® (idebenone) is currently under review by the European Medicines Agency. Santhera has an option to license vamorolone, a first-in-class anti-inflammatory drug candidate with novel mode of action, currently investigated in a pivotal study in patients with DMD to replace standard corticosteroids. The clinical stage pipeline also includes POL6014 to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases, as well as omigapil and an exploratory gene therapy approach targeting congenital muscular dystrophies. Santhera out-licensed ex-North American rights to its first approved product, Raxone® (idebenone), for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. For further information, please visit www.santhera.com.

Raxone® and Puldysa® are trademarks of Santhera Pharmaceuticals.

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