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A conference call will be held today at 13:00 CEST, 12:00 BST, 07:00 EDT. Details are at the end of this statement.

Santhera Announces Financial Results for the First Half-Year 2019

Pratteln, Switzerland, September 3, 2019 – Santhera Pharmaceuticals (SIX: SANN) reports first half-year results as of June 30, 2019, and provides an update on its pipeline and strategic focus.

Thomas Meier, PhD, Chief Executive Officer of Santhera, said: "Following a series of transactions which strategically reshaped the direction of the Company, we are well positioned to advance our long-term growth strategy of focusing on medicines to treat neuromuscular and pulmonary diseases. For 2019, we are on track to achieve our strategic objectives, having filed for conditional marketing authorization of Puldysa® (idebenone) in Europe for the preservation of respiratory function in Duchenne muscular dystrophy (DMD) patients. We expect an opinion from the EMA's Committee for Medicinal Products for Human Use (CHMP) mid-2020. Our goal is to help all DMD patients, irrespective of causative mutations, disease stage or age. We were therefore very pleased about the recent publication by ReveraGen of positive Phase IIa-extension study results with vamorolone in the journal *Neurology*. These data together with previous publications provide proof-of-concept that vamorolone can improve gross muscle function outcomes in young patients with DMD and indicate a better tolerability profile than standard corticosteroids."

"During the period, we announced an exclusive agreement with Chiesi Group to out-license Raxone® for the treatment of Leber's hereditary optic neuropathy (LHON) for a total consideration of up to EUR 93 million with an upfront payment of EUR 44 million. In monetizing this commercial product, we have strengthened our financial position to focus on upcoming regulatory milestones and commercialization activities for our neuromuscular and pulmonary product candidates, which are core to our long-term growth strategy."

Financial highlights:

- 1H-2019 sales on track with CHF 18.3 million, an increase of 14% compared to 1H-2018
- Operating expenses of CHF 38.2 million (1H-2018: CHF 39.9 million)
- Operating result of CHF –22.4 million (1H-2018: CHF –26.3 million) leading to a net result of CHF –26.9 million (1H-2018: CHF –27.4 million)
- Cash and cash equivalents of CHF 43.7 million (August 31, 2019)
- Full-year sales guidance of CHF 25-27 million

First half-year overview:

Regulatory momentum for Puldysa in DMD

In June, the European Medicines Agency (EMA) validated Santhera's application for conditional marketing authorization (CMA) for Puldysa (idebenone) in the treatment of respiratory dysfunction in patients with Duchenne muscular dystrophy (DMD) who are not using glucocorticoids. The review

process by the EMA's CHMP has begun and the Company expects an opinion by the CHMP around mid-2020. In addition, the UK's Medicines and Healthcare products Regulatory Agency (MHRA) renewed its positive scientific opinion for idebenone for patients with DMD in respiratory function decline who are not taking glucocorticoids, under the Early Access to Medicines Scheme (EAMS).

Long-term data with Puldysa

In February, Santhera announced the results of its SYROS study, which investigated long-term efficacy with idebenone in slowing respiratory function loss in patients with DMD. The study demonstrated that long-term treatment with idebenone consistently contributed to the preservation of respiratory function for up to 6 years in a real-world setting. This long-term data further supports the potential for idebenone to positively modify the course of respiratory function decline and delay the time to clinically relevant milestones.

Strong Raxone performance underscores Santhera's commercial expertise

Net sales of Raxone in Europe amounted to CHF 18.3 million (1H-2018: CHF 16.0 million) which corresponds to a 14% increase year-on-year, in line with the previous full-year product sales guidance. In May, the Company announced that it had entered into an exclusive license agreement with Chiesi Group for Raxone for the treatment of LHON, for a total consideration of up to EUR 93 million. With the closing at the end of July, Santhera has now transferred all rights to Chiesi Group for the development, commercialization and distribution of Raxone for the treatment of LHON and any other potential ophthalmological indications for all territories worldwide except the US and Canada. After the closing and for an interim period, Santhera will provide support services to Chiesi Group to enable a seamless handover of the business and will continue to commercialize Raxone for LHON in France.

Positive study data with vamorolone published– pivotal study enrolling

Last month, positive data from 6-month Phase IIa-extension study (VBP15-003) with vamorolone in DMD were published by ReveraGen in Neurology [1]. The data demonstrated dose-related improvement of gross muscle function in patients with DMD treated with vamorolone. Vamorolone was reported to be safe and well tolerated up to the highest dose tested (6.0 mg/kg/day). Biomarker data indicated reduced occurrence of side effects typical for traditional corticosteroid drugs. Based on these data, vamorolone has potential to replace standard corticosteroids currently used in patients with DMD.
ReveraGen is presently enrolling the Phase IIb VISION-DMD study [2] (VBP15-004; clinicaltrials.gov: NCT03439670), designed as a pivotal efficacy and safety trial. The study is expected to be fully enrolled by the end of 2019. Accordingly, the 6-month randomized placebo-controlled treatment period would end by mid-2020, followed by data analysis. NDA submission could be towards year end 2020.

Collaboration to advance gene therapy research for rare neuromuscular disease

Santhera initiated a collaboration with the Biozentrum of the University of Basel to advance gene therapy research for the treatment of LAMA2-deficient congenital muscular dystrophy (LAMA2 MD or MDC1A). The preclinical research collaboration builds on previous work with omigapil, which was recently studied in a Phase I clinical trial and could act complementary. The program is supported by public funding for innovation in Switzerland through a grant from Innosuisse – the Suisse Innovation Agency.

Continued investment in clinical development

Santhera is running several late-stage clinical trials, among them SIDEROS, the largest ever conducted study in patients with DMD. The SIDEROS study is 84% recruited and positive outcome of the study will form the basis for NDA submission for patients with DMD irrespective of glucocorticoid status, currently expected in 2H-2021. In addition, the preparation of the MAA for Puldysa, remaining post-approval clinical work for Raxone in LHON and increased clinical development work with POL6014 entailed slightly higher development expenses of CHF 19.3 million (+2% year-on-year). Overall, operating expenses showed a small decline (–4%) driven by lower expenses for commercial activities (–10%).

Liquidity base allows for the continuation of the strategy as planned

In April, Santhera raised CHF 7.1 million by the placement of 500,000 shares. As of the end of June 2019, Santhera had cash and cash equivalents of CHF 12.7 million (December 31, 2018: CHF 22.0 million). Together with the net proceeds from the initial payment from Chiesi Group following closing of the licensing transaction, liquid funds amounted to CHF 43.7 million (August 31, 2019), allowing the Company to proceed with its clinical trial program and regulatory filings as planned.

Outlook and Guidance

Based on the performance in the first half-year of 2019, the Company expects to achieve annual net sales with Raxone in the currently approved indication LHON of CHF 25-27 million in 2019, taking into account that Chiesi Group has taken over commercial sales for Raxone from August in all European countries except France. The operational priorities for 2019 are the preparation of European market entry with Puldysa in DMD in 2020, completing enrollment into the DMD SIDEROS trial to support the planned US-submission of Puldysa in DMD, and advancing the other clinical stage candidates in the pipeline, particularly vamorolone and POL6014.

References:

[1] Hoffman EP et al. (2019). Vamorolone trial in Duchenne muscular dystrophy shows dose-related improvement of muscle function. Neurology 2019. https://n.neurology.org/lookup/doi/10.1212/WNL.0000000000008168
[2] https://vision-dmd.info/

Half-year Report

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

Conference Call

Santhera will host a conference call on September 3, 2019 at 13:00 CEST / 12:00 BST / 07:00 EDT. Thomas Meier, PhD, CEO of Santhera, will discuss the half-year 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 Half-year Financial Information

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

Condensed consolidated income statement (reviewed, IFRS, for half-year ended June 30, in CHF thousands)	**1H-2019**	1H-2018
Net sales	**18,315**	16,027
Cost of goods sold (of which amortization intangible assets: 2019 –1,519 / 2018 –1,519)	**–2,557**	–2,441
Development	**–19,325**	–18,854
Marketing and sales	**–11,611**	–12,921
General and administrative	**–7,206**	–8,051
Operating expenses	**–38,208**	–39,883
Operating result	**–22,434**	–26,297
Financial result	**–4,065**	–961
Income taxes	**–401**	–93
Net result	**–26,900**	–27,351
Basic and diluted loss per share (in CHF)	**–2.47**	–4.25

Condensed consolidated balance sheet (IFRS, in CHF thousands)	**June 30, 2019** **(reviewed)**	Dec. 31, 2018 (audited)
Cash and cash equivalents	**12,698**	21,971
Other current assets	**25,774**	21,112
Noncurrent assets	**67,620**	67,211
Total assets	**106,092**	110,294
Equity	**9,254**	27,829
Noncurrent liabilities	**69,609**	62,756
Current liabilities	**27,229**	19,709
Total equity and liabilities	**106,092**	110,294

Condensed consolidated cash flow statement (reviewed, IFRS, in CHF thousands)	**2019**	2018
Operating cash flow for the half-year ended June 30	**–20,219**	–22,154
Investing cash flow for the half-year ended June 30	**1,448**	137
Financing cash flow for the half-year ended June 30	**9,465**	–1,107
Cash and cash equivalents at January 1	**21,971**	45,195
Cash and cash equivalents at June 30	**12,698**	22,082
Net change in cash and cash equivalents	**–9,273**	–23,113

Share capital (number of shares with par value of CHF 1)	**June 30,2019** **(reviewed)**	Dec. 31, 2018 (audited)
Shares issued	**11,164,563**	10,664,563
Conditional capital for equity rights plans	**687,552**	687,552
Conditional capital for conversion 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 leading Duchenne muscular dystrophy (DMD) franchise. 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 dissociative steroid 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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