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Santhera will hold a conference call 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 2020 and Updates on Corporate Progress

- **Net revenues of CHF 7.8 million, operating expenses reduced by 16%, net result of CHF -31.8 million**
- **Closing of financing arrangements in April and July to provide in total up to CHF 32 million with the option to increase to up to CHF 44 million**
- **Puldysa® (idebenone) expecting CHMP opinion in Q4-2020 following inclusion of data from SIDEROS trial interim analysis**
- **Vamorolone VISION-DMD study nearing full enrollment and 6-month topline results expected in Q2-2021**
- **Worldwide rights to vamorolone in Duchenne muscular dystrophy and all other indications obtained in September 2020**

Pratteln, Switzerland, September 8, 2020 – Santhera Pharmaceuticals (SIX: SANN) announces the Company's financial results for the first half-year 2020 and provides an update on progress made in advancing its lead compounds for the treatment of Duchenne muscular dystrophy (DMD).

"Our primary focus continues to be on advancing our business towards several significant inflection points with our DMD drug candidates, Puldysa and vamorolone, and securing sufficient funds to enable the ongoing implementation of our growth plans," said **Dario Eklund, Chief Executive Officer of Santhera**. "After having successfully secured all rights to vamorolone, we now have a DMD franchise that has the potential to address the unmet medical needs of a broad patient population. For Puldysa, we intend to present the data from the planned interim analysis of the SIDEROS study to the European regulators and, subject to a positive CHMP opinion in the fourth quarter 2020, aim at a launch in Europe in the first quarter 2021. Whilst for vamorolone, our second DMD drug candidate, we now expect read-out of topline data from the pivotal VISION-DMD trial in the second quarter 2021 due to the Covid-19-related delays."

He added: "Our sincere thanks go to all clinical trial participants, their families and carers, for their commitment to advancing our drug candidates in minimizing the delays under the severe constraints imposed by the Covid-19 pandemic. Likewise, we are also appreciative of the investor support which allowed us to obtain additional financing under challenging market conditions. I am confident that the upcoming value-enhancing inflection points will trigger further interest in Santhera and facilitate the raising of additional finance to support ongoing development and commercialization activities."

KEY FINANCIALS

- Net revenues from product sales of CHF 7.8 million
- Operating expenses of CHF -31.9 million, reduced by 16%
- Net result of CHF -31.8 million (1H-2019: CHF -26.9 million)
- Cash and cash equivalents of CHF 19.4 million (June 30, 2020)
- Operating cash flow CHF -19.8 million

Sales of Raxone phasing out after transfer of business to Chiesi Group

In the first six months 2020, Santhera reported revenue from contracts with customers of CHF 7.8 million (1H-2019: CHF 18.3 million). The majority of this revenue reflects sales of Raxone for the treatment of Leber's hereditary optic neuropathy (LHON) in France, where Santhera continues to market the product following the out-licensing and transfer to Chiesi Group. As previously announced, 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 CHF 105 million of which CHF 46.4 million was recognized as revenue in 2019.

Operating cost reductions of 16%

As a result of rigorous cost saving measures, total operating expenses for the first half 2020 decreased by 16% year-on-year to CHF 31.9 million (1H-2019: CHF 38.2 million). Development expenses were down 8% to CHF 17.7 million (1H-2019: CHF 19.3 million) and reflect primarily costs for ongoing late stage clinical studies, including the Phase 3 SIDEROS trial in DMD, and efforts associated with the pending marketing authorization application for Puldysa for DMD in Europe. Marketing and sales expenditures declined by 42% to CHF 6.8 million (1H-2019: CHF 11.6 million) as commercial activities were rescheduled and aligned with the expected market entry of Puldysa in the first quarter 2021. General and administrative expenses of CHF 7.2 million were unchanged year-on-year (1H-2019: CHF 7.2 million). Overall, the Company reported an operating result of CHF -25.9 million (1H-2019: CHF -22.4 million). Decreased revenues following the out-licensing were partially compensated by cost reduction measures, resulting in a net result of CHF -31.8 million (1H-2019: CHF -26.9 million).

Recent financings provide additional liquidity

As of June 30, 2020, cash and cash equivalents amounted to CHF 19.4 million (December 31, 2019: CHF 31.4 million). In recent months, Santhera successfully secured additional funds which provide a runway to advance value-enhancing developments and pre-commercialization activities for the neuromuscular compounds Puldysa and vamorolone.

In April, Santhera entered into a financing arrangement with IRIS (France) in the initial gross amount of up to CHF 12 million over 12 months, with the extension option for another CHF 12 million over the following 12 months. As of June 30, 2020, the Company had received gross proceeds of CHF 8 million from the arrangement with IRIS. Post period end, on July 14, 2020, Santhera closed an up to CHF 20 million financing facility with a fund managed by Highbridge Capital Management, LLC, an existing investor in the Company. As of September 7, 2020, the Company has received the first tranche of CHF 7.5 million from this arrangement with Highbridge Capital Partners, LLC, CHF 5 million of which has been repaid via the exchange for Company shares. Additional tranches of up to CHF 12.5 million may be drawn contingent on the achievement of milestones. The conditional and authorized capital approved in April

2020 by the Annual General Meeting was in part used to serve these equity-linked financing arrangements.

In parallel, the Company is evaluating a restructuring of the CHF 60 million Senior Unsecured Convertible Bonds with a February 2022 maturity.

For ongoing development activities, the preparation for commercial launch of Puldysa and the payments due following the exercise of the sub-license option for vamorolone, Santhera will require further additional funding.

From September 21, 2020, Santhera will be included in both of the SIX Swiss Exchange healthcare indices: SXI Life Sciences®, which includes pharmaceutical, biotechnology and medical technology companies, and its more narrowly defined sub-index SXI Bio+Medtech®, focused on biotech and medtech companies, which is expected to further enhance investor visibility.

PIPELINE MILESTONES AND PROGRESS

With Puldysa and vamorolone, Santhera is building a complementary DMD product portfolio. The Company expects the availability of both vamorolone and Puldysa to address the medical needs of DMD patients, from early to late disease stages, irrespective of age, underlying dystrophin mutation or ambulatory status. Santhera's pipeline priorities for the remainder of 2020 continue to focus on advancing its neuromuscular franchise in DMD, with Puldysa and vamorolone, towards value-enhancing inflection points:

- September 2, 2020: Santhera obtains worldwide rights to vamorolone in all indications
- Q4-2020: Interim analysis of SIDEROS study and inclusion of data into regulatory dossier
- Q4-2020: CHMP opinion on marketing authorization application for Puldysa in DMD in Europe
- Q1-2021: Launch of Puldysa in first European markets
- Q2-2021: Read-out of topline data of pivotal Phase 2b trial for vamorolone in DMD
- Q4-2021: NDA (new drug application) filing in the US for vamorolone in DMD

Puldysa—ahead of DMD SIDEROS interim analysis and CHMP opinion

Puldysa highlights in the first half-year were the renewal of the Early Access to Medicines Scheme (EAMS) scientific opinion in the UK and the completion of enrollment into the Phase 3 SIDEROS study in DMD. With the EAMS renewal for another year, the UK's Medicines and Healthcare products Regulatory Agency (MHRA) has confirmed its positive scientific opinion for idebenone enabling continued pre-approval access to idebenone for patients with DMD in respiratory function decline who are not taking glucocorticoids. In May, Santhera announced full recruitment into the SIDEROS study and its intention of conducting an interim analysis by the independent Data and Safety Monitoring Board (DSMB), subject to approval of the necessary study protocol amendment. If positive results are shown, the additional clinical data will be included into the European conditional marketing authorization application for which the Company now expects a CHMP opinion in the fourth quarter of 2020. Simultaneously, preparations for market entry will be advanced to allow for a launch in Europe in the first quarter 2021, subject to timely product approval. In the US, a positive outcome of the interim analysis, followed by an early completion of the SIDEROS study, could allow acceleration of a regulatory filing with the FDA by approximately one year. Santhera estimates the peak sales potential for Puldysa to be in excess of USD 500 million in the US and the largest five EU countries.

Vamorolone—encouraging new long-term clinical data and VISION-DMD nearly fully enrolled

In June, Santhera's partner ReveraGen completed a long-term, open-label 24-month extension study (VBP15-LTE) in patients with DMD. Enrolled in this study were patients who had previously completed a 6-month dose escalation study (VBP15-003) which demonstrated dose-dependent improvement in timed function tests and good tolerability. Taken together, ReveraGen has now obtained safety and efficacy data with vamorolone over a period of 2.5 years in 41 boys with DMD. Currently ongoing is the pivotal VISION-DMD study which compares the efficacy and tolerability profile of vamorolone versus placebo and prednisolone to determine whether vamorolone improves muscle strength and function compared to placebo and whether it has less side effects compared to prednisolone, thereby potentially making it a valuable alternative to standard corticoid treatments. The next study milestone will be the soon expected full enrollment into the study followed by topline 6-month data readout anticipated in the second quarter of 2021 which, if positive, could allow for an NDA filing in the fourth quarter of 2021.

Vamorolone—worldwide rights for all indication obtained

On September 2, 2020, Santhera announced the signing of agreements with Idorsia (SIX: IDIA) and ReveraGen BioPharma Inc., making Santhera a direct license holder of vamorolone [1]. Under the agreements, Santhera has obtained an exclusive license from ReveraGen, the originator of vamorolone, for all indications worldwide. The agreements create further value for Santhera through the transfer of rights for the previously excluded markets Japan and South Korea, the right to grant sublicenses and a share in the expected Priority Review Voucher. Under the amended terms, Santhera expects a reduction in cash outflow in the range of USD 18-24 million in the next 12-18 months. Santhera estimates the peak sales potential for vamorolone for the DMD indication alone to be in excess of USD 500 million in the US and the largest five EU countries.

Progress made with earlier pipeline projects

Santhera is advancing its Phase 1b study with clinical stage candidate lonodelestat for cystic fibrosis. The compound's potential as a therapeutic intervention for COVID-19-related acute respiratory distress syndrome (ARDS) is investigated in preclinical research by Cold Spring Harbor Laboratory (CSHL) under a collaboration agreement. The pipeline also includes a discovery-stage gene therapy approach for which Santhera signed agreements with University Basel and Rutgers University as part of its program to advance gene therapy research for the treatment of LAMA2-deficient congenital muscular dystrophy (LAMA2 MD or MDC1A). As an ongoing business development activity, Santhera is evaluating further diversification of its platform-type pipeline products vamorolone and lonodelestat into additional indications which may include partnering with other companies.

Outlook

The operational priorities for Santhera in the second half of 2020 are the preparation for European market entry of Puldysa in DMD in early 2021, advancing vamorolone towards the VISION-DMD top-line data readout and securing additional funding to allow the Company to pursue its operations as planned.

Reference:

[1] Press release "Santhera Exercises Option to Obtain Worldwide Rights to Vamorolone in Duchenne Muscular Dystrophy and All Other Indications", September 2, 2020, accessible here.

2020 Half-year Financial Information

Santhera's 2020 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-2020**	1H-2019
Net sales	**6,133**	18,315
Net sales to licensing partner	**1,642**	0
Revenue from contracts with customers	**7,775**	18,315
Cost of goods sold (of which amortization intangible assets: 1H-2020 -1,519 / 1H-2019 -1,519)	**-2,114**	-2,557
Development	**-17,688**	-19,325
Marketing and sales	**-6,766**	-11,611
General and administrative	**-7,209**	-7,206
Operating expenses	**-31,911**	-38,208
Operating result	**-25,893**	-22,434
Financial result	**-5,573**	-4,065
Income taxes	**-361**	-401
Net result	**-31,827**	-26,900
Basic and diluted loss per share (in CHF)	**-2.78**	-2.47

Condensed consolidated balance sheet (IFRS, in CHF thousands)	**June 30, 2020** **(reviewed)**	Dec. 31, 2019 (audited)
Cash and cash equivalents	**19,353**	31,358
Other current assets	**17,201**	17,897
Noncurrent assets	**63,520**	65,796
Total assets	**100,074**	115,051
Equity	**210**	21,247
Noncurrent liabilities	**69,894**	69,840
Current liabilities	**29,970**	23,964
Total equity and liabilities	**100,074**	115,051

Condensed consolidated cash flow statement (reviewed, IFRS, in CHF thousands)	**2020**	2019
Operating cash flow for the half-year ended June 30	**-19,795**	-20,219
Investing cash flow for the half-year ended June 30	**1,506**	1,448
Financing cash flow for the half-year ended June 30	**6,405**	9,465
Cash and cash equivalents at January 1	**31,358**	21,971
Cash and cash equivalents at June 30	**19,353**	12,698
Net change in cash and cash equivalents	**-12,005**	-9,273

Share capital (number of shares with par value of CHF 1)	**June 30, 2020** **(reviewed)**	Dec. 31, 2019 (audited)
Shares issued	**13,185,063**	11,165,063
Conditional capital for equity rights	**687,052**	687,052
Conditional capital for convertible rights	**4,800,000**	2,500,000
Authorized capital	**4,630,000**	3,000,000

Half-year Report

The Santhera Half-year Report 2020 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 8, 2020 at 13:00 CEST / 12:00 BST / 07:00 EDT. CEO Dario Eklund and CFO Andrew Smith will discuss the 2020 half-year financial results and recent 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

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 from early to late disease stages, irrespective of causative mutations, ambulatory status or age. A marketing authorization application for Puldysa® (idebenone) is currently under review by the European Medicines Agency. Santhera has an exclusive license for all indications worldwide to vamorolone, a first-in-class anti-inflammatory drug candidate with novel mode of action, currently investigated in a pivotal study in patients with DMD as an alternative to standard corticosteroids. The clinical stage pipeline also includes lonodelestat (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. Further information at www.santhera.com.

Puldysa® and Raxone® are trademarks of Santhera Pharmaceuticals.

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