Santhera Pharmaceuticals Holding AG
Hohenrainstrasse 24, 4133 Pratteln, Switzerland

Phone: +41 61 906 89 50 | Fax: +41 61 906 89 51
www.santhera.com

A conference call will be held today at 14:00 CEST, 13:00 BST, 08:00 EDT. Details are at the end of this statement.

Santhera Reports 2020 Annual Results

- **Revenue from contracts with customers of CHF 15.0 million**
- **Operating result of CHF -53.1 million (2019: CHF -10.4 million) and net result of CHF -67.7 million (2019: CHF -19.0 million)**
- **Cash and cash equivalents of CHF 12.4 million as of December 31, 2020**
- **Refreshed clinical and operational strategy as the basis for future growth**
- **Strong progress in advancing lead compound vamorolone towards regulatory submission for Duchenne muscular dystrophy (DMD)**

Pratteln, Switzerland, April 29, 2021 – Santhera Pharmaceuticals (SIX: SANN) announces the Company's audited financial results for 2020 and reports on progress in advancing its lead compound for the treatment of Duchenne muscular dystrophy (DMD), vamorolone, towards regulatory submission.

"Santhera started 2021 with the strong foundation of a refreshed clinical and operational strategy focused on vamorolone, which we believe can provide significant value to patients, caregivers, and ultimately shareholders. It represents the key foundation for the future of Santhera, which continues to focus on Duchenne muscular dystrophy (DMD) and other rare diseases.," said **Dario Eklund, CEO of Santhera**. "Vamorolone is currently being developed for early stage DMD patients and recent encouraging data leads us to conclude that vamorolone could emerge as a foundational therapy in DMD for all patients irrespective of gene mutation and as a promising alternative to existing corticosteroids. Based on the collective clinical experience so far, we look forward to the readout of the 6-month top-line data from the pivotal VISION-DMD study, the next value enhancing inflection point. Subject to a positive outcome in Q2-2021, we will push ahead with the filing of a New Drug Application (NDA) with the US FDA and will step up preparations for market entry."

KEY FINANCIALS

- Net revenue from contracts with customers of CHF 15.0 million
- Operating expenses of CHF -58.3 million, reduced by 28%
- Net result of CHF -67.7 million (2019: CHF -19.0 million)
- Cash and cash equivalents of CHF 12.4 million (December 31, 2020)
- Operating cash flow CHF -43.5 million

2020 full-year net revenues in line with expectations

In 2020, Santhera reported net revenue from its product Raxone of CHF 15.0 million (2019: CHF 75.4 million including CHF 46.4 million out-licensing income). This predominantly reflects sales of Raxone for the treatment of Leber's hereditary optic neuropathy (LHON) in France where Santhera still markets the product following the out-licensing to Chiesi Group in August 2019 outside of North America (2019: 24 European countries, with the majority of sales reached in France and Germany). The reduction on prior

year of CHF 60.4 million reflects the full year impact of territories out-licensed to Chiesi Group as well as the inclusion of the upfront milestone recognized in 2019.

Cost of goods sold
Cost of goods sold were CHF 10.4 million (2019: CHF 5.5 million). The increase was primarily due to a one-off impairment of CHF 6.0 million related to the discontinuation of Puldysa, partially offset by the full year impact of out-licensed Raxone activities.

Operating expenses
With CHF 58.3 million, total operating expenses were significantly lower year-on-year (2019: CHF 80.7 million). The decrease in development expenses to CHF 34.2 million (2019: CHF 41.2 million) was primarily related to the discontinuation of the Puldysa development in Q4-2020. Marketing and sales decreased to CHF 11.5 million (2019: CHF 20.1 million) primarily as a result of the full year impact of out-licensed Raxone activities and termination of the Puldysa program. General and administrative purposes decreased to CHF 12.4 million (2019: CHF 19.2 million) as support activities were reduced and the European subsidiaries were closed.

During the year, the Puldysa development program was terminated which necessitated an organizational restructuring. During 2020, cost of goods recorded an inventory impairment of CHF 6.0 million and operating expenses included Puldysa related costs of CHF 11.4 million which are both non-recurring. In addition, the organizational restructuring, resulting in a reduction of headcount by over 50%, is expected to reduce staff costs by approximately CHF 10.0 million in future periods. Together, these represent costs incurred in the year of CHF 27.4 million not expected to occur in future periods. Going forward, the Company continues to reduce cost in other areas, however, in the event of positive upcoming vamorolone results, the Company expects to increase certain costs to support approval and pre-commercialization activities. Other costs incurred to carry out post-marketing study obligations for Raxone which are expected to be completed during 2021 and support the ongoing development of lonodelestat, which announced positive Phase 1 results in March 2021.

Financial income and expenses
Net financial income and expenses were CHF 14.4 million (2019: CHF 8.0 million). The increase of CHF 6.4 million was primarily due to the cost of raising additional funding during the year as well as the effect of currency gains & losses and the effect of derivative accounting adjustments.

Net result
The net result was a loss of CHF 67.7 million (2019: CHF 19.0 million), an increase of CHF 48.7 million on the prior year. The widening of the loss was predominantly the result of lower revenue of CHF 60.4 million following the out-licensing of Raxone which was only partially offset by cost reductions and also reflected the costs of development and termination of Puldysa.

Cash flow and cash balance
Cash used in operating activities was CHF 43.5 million (2019: inflow CHF 2.6 million) an increase of CHF 40.9 million which was mainly due to the reduction in revenue of CHF 60.4 million following out-licensing of Raxone being offset by other reduction in expenses.

The cash and cash equivalents at December 31, 2020 were CHF 12.4 million (2019: CHF 31.4 million).

Financial outlook

Santhera is still commercializing Raxone for LHON in France in a transitional phase and, as previously communicated, sales of the product are declining. From August 2021 onwards, as Raxone is expected to no longer be on the list of reimbursed products in France, the Company will continue to supply medication in the interest of patients, but does not expect to generate further products sales. Over the coming months, post-authorization studies will be completed upon which Santhera will resume discussions on reimbursement with the French authorities. Subject to the achievement of certain commercial milestones for Raxone, Santhera is entitled to contingent variable near- to mid-term milestone payments from Chiesi Group of up to EUR 49 million.

Currently, the Company has a limited cash runway to the third quarter 2021 and thus material uncertainties remain as to the Company's ability to continue as a going concern until December 31, 2021. Ongoing development activities and increase in pre-commercialization activities relating to vamorolone will require substantial additional funding, particularly in the latter part of 2021. Executing the Company's strategy depends on further funding to ensure the continuation of its operations through December 31, 2021.

As reported in the 2019 annual report, released in March 2020, Santhera had sufficient funds to mid-2020. The Company entered into financing arrangements with IRIS and Highbridge Capital which provided additional funding. As a result of the termination of the Puldysa program in 2020, the Company ended 2020 with a limited cash run-way and the anticipation to raise additional finance in the event of positive vamorolone 6-month study results expected in the second quarter of 2021. Following the initiatives taken during the fourth quarter of 2020, cash flow from operating activities had been significantly reduced and in February 2021 an amendment to the agreement with Highbridge was entered into which provided additional funding and extended the cash runway further into the third quarter of 2021.

Given the overall liquidity position and the requirement to raise additional funding during 2021, the Company commenced a restructuring of the CHF 60 million convertible bond maturing in February 2022. The process for this is in the final stages and is expected to result in an exchange of approximately 75% to a new bond with an extended maturity to August 2024, thereby reducing the amount maturing in February 2022 to approximately CHF 15.2 million.

Cash and cash equivalents as at April 27, 2021 were CHF 11.7 million, in addition CHF 6 million, subject to certain drawdown conditions being met, is available for drawdown under the Highbridge agreements.

Previously, the Company held an Extraordinary General Meeting on March 18, 2021, where the shareholders approved additional authorized and conditional share capital that would be required in the event of conversion of the convertible bond as per the new terms as well as to allow for some additional financing.

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. Potential requirements and sources will be further evaluated following the outcome of the upcoming vamorolone 6-month results. At the forthcoming Annual General Meeting on June 22, 2021, the Board plans to make corresponding proposals.

PIPELINE MILESTONES AND PROGRESS

Santhera's pipeline priorities for 2021 continue to focus on advancing vamorolone towards regulatory submission and progressing lonodelestat:

- Q1-2021: Phase 1 program with lonodelestat in cystic fibrosis completed
- Q2-2021: 6-month top-line data readout from VISION-DMD study of vamorolone
- Q1-2022: NDA (new drug application) filing in the US for vamorolone in DMD
- Q2-2022: MAA (marketing authorization application) filing in Europe for vamorolone in DMD

Vamorolone—Santhera's strategic near- and mid-term pipeline priority

In 2020, the vamorolone development program made significant progress with core and extension studies generating a wealth of encouraging new clinical data, shedding light on the compound's novel mode of action, its efficacy, differentiated safety and favorable tolerability profile. The molecular distinctions of vamorolone compared to standard corticosteroids are thought to explain the unique properties of the drug candidate by dissociating efficacy from typical steroid safety-related concerns that limit their use and lead to high levels of treatment discontinuation. Recently published data from open-label studies (VBP15-003 and VBP15-LTE) evaluated the long-term safety, tolerability and efficacy of vamorolone in patients with DMD and showed improvements from baseline with vamorolone on all measured motor functions through the 18-month follow-up period. These improvements were comparable to those seen in historic corticosteroid-treated patients. Equally important, vamorolone did not show stunting of growth, as seen with deflazacort and prednisone, and also showed fewer physician-reported adverse events such as mood disturbance, excessive hair growth, and Cushingoid appearance. These findings were confirmed in the Phase 2a long-term treatment data which demonstrate a maintenance of treatment effect, equivalent to a delay of about two years in decline for time to stand (TTSTAND) velocity, and confirm safety and tolerability benefits of vamorolone over the 2.5-year follow up period. On this basis, Santhera believes that vamorolone could emerge as a foundational therapy in DMD for all patients irrespective of gene mutation and a promising alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD. The next value enhancing inflection point is the readout of topline data from the pivotal VISION-DMD study, expected in Q2-2021. If positive, this would pave the way for a US NDA submission in Q1-2022 and in the EU, upon availability of positive 12-month data, in Q2-2022.

Lonodelestat—positive results in early phase cystic fibrosis trial

Likewise, lonodelestat, a potent and selective peptide inhibitor of human neutrophil elastase (hNE) in development to treat cystic fibrosis (CF), made good progress. Neutrophil elastase is an enzyme associated with tissue inflammation, leading to degradation of the lung tissue in cystic fibrosis and several other chronic inflammatory conditions of the lung where neutrophils play a prominent role in the disease process. In December 2020, Santhera completed a double-blind, placebo-controlled multiple ascending dose Phase 1b study in patients with CF which assessed the safety, tolerability, pharmacokinetics and pharmacodynamics of orally inhaled daily doses of lonodelestat for up to four weeks. The study established a safe dose regimen and provided promising data on the safety of

lonodelestat. Furthermore, the study demonstrated that lonodelestat reaches its intended target in the lung and achieves the desired effect of complete inhibition of elastase without any drug/metabolite accumulation. On this basis, Santhera will now be optimizing the further clinical development program to advance lonodelestat for the treatment of CF and potentially for other inflammatory pulmonary conditions, whether acute or chronic.

Partnering for platform-like molecules and early stage pipeline

Santhera has started the pursuit of partnering opportunities for vamorolone in additional indications outside DMD and in geographies outside the US and Europe which could result in significant future non-dilutive income streams. Preclinical data with vamorolone have already been obtained in in vitro and in vivo models for asthma, multiple sclerosis, inflammatory bowel disease, rheumatoid arthritis, critical illness myopathy, and brain tumor. In some of these diseases, the prescription of standard glucocorticoids is limited due to detrimental side-effects. In parallel, the Company is proactively pursuing collaborations with partners to assess and exploit the potential of lonodelestat in other pulmonary diseases beyond CF and for its undertakings in gene therapy.

Further information about Santhera's development pipeline is available in the **Annual Report 2020** which can be viewed here.

Statement on the impact of the COVID-19

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.

Outlook

The operational priorities for Santhera in 2021 are the preparation for a US regulatory filing, subject to a positive 6-month top-line data readout of the VISION-DMD study, and securing additional funding to allow the Company to pursue its operations as planned.

Annual Report

The Santhera Annual 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 April 29, 2021 at 14:00 CEST / 13:00 BST / 08:00 EDT. CEO Dario Eklund and CFO Andrew Smith will discuss the 2020 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

2020 Full-year Financial Information

Santhera's 2020 Annual Report see www.santhera.com/investors-and-media/investor-toolbox/financial-reports.

Condensed consolidated income statement (IFRS, in CHF thousands)	**2020**	2019
Net sales	**11,252**	27,890
Revenue from out-licensing transactions	**1,597**	46,370
Net sales to licensing partner	**2,159**	1,116
Revenue from contracts with customers	**15,008**	75,376
Cost of goods sold (of which amortization intangible assets: 2020 -3,039 / 2019 -3,039)	**-10,431**	-5,450
Development	**-34,228**	-41,244
Marketing and sales	**-11,474**	-20,096
General and administrative	**-12,440**	-19,184
Operating expenses	**-58,347**	-80,652
Operating result	**-53,076**	-10,442
Financial result	**-14,380**	-7,952
Income taxes	**-203**	-579
Net result	**-67,659**	-18,973
Basic and diluted loss per share (in CHF)	**-5.08**	-1.73

Condensed consolidated balance sheet (IFRS, in CHF thousands)	**2020**	2019
Cash and cash equivalents	**12,411**	31,358
Other current assets	**5,312**	17,897
Noncurrent assets	**70,964**	65,796
Total assets	**88,687**	115,051
Equity	**-6,354**	21,247
Noncurrent liabilities	**65,972**	69,840
Current liabilities	**29,069**	23,964
Total equity and liabilities	**88,687**	115,051

Condensed consolidated cash flow statement (IFRS, in CHF thousands)	**2020**	2019
Cash flow from/(used) in operating activities	**-43,510**	2,595
Cash flow from/(used) in investing activities	**-10,802**	2,789
Cash flow from financing activities	**23,150**	4,129
Cash and cash equivalents at January 1	**31,358**	21,971
Cash and cash equivalents at December 31	**12,411**	31,358
Net change in cash and cash equivalents	**-18,947**	9,387

Share capital (number of shares with par value of CHF 1)	**2020**	2019
Shares issued	**19,429,696**	11,165,063
Conditional capital for equity rights	**687,052**	687,052
Conditional capital for convertible rights	**1,104,658**	2,500,000
Authorized capital	**2,080,709**	3,000,000

Related documents

Notice of a Repurchase Offer and Preliminary Issuance and Listing Prospectus Regarding the New Bonds: https://www.santhera.com/investors-and-media/investor-toolbox/bond-exchange-offering

Forms to cast bondholder votes are available here.

Invitation to the Bondholders' Meeting (March 8, 2021), the EGM (March 18, 2021) and accompanying documents: http://www.santhera.com/investors-and-media/investor-toolbox/share-bondholder-meetings

Corporate calendar

June 22, 2021 Annual General Meeting

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 has an exclusive license for all indications worldwide to vamorolone, a first-in-class dissociative steroid 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 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® is a trademark of Santhera Pharmaceuticals.

For further information please contact:
public-relations@santhera.com or
Eva Kalias, Head External Communications
Phone: +41 79 875 27 80
eva.kalias@santhera.com

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