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Santhera Announces Corporate Update and Proposal to Strengthen Capital Structure

- **Pivotal 6-month data readout for vamorolone in Duchenne muscular dystrophy (DMD) expected in the second quarter of 2021**
- **Organization streamlined with a focus on cost savings and progressing vamorolone**
- **Preliminary unaudited result for 2020: net sales CHF 15.0 million; net loss CHF 71.2 million; cash on hand as of December 31, 2020, CHF 12.4 million**
- **Upsized financing from fund managed by Highbridge Capital Management, LLC (an existing investor in the Company), to satisfy liquidity needs through the next value-inflection point**
- **Further strengthening of Santhera's capital structure through a proposed convertible bond restructuring supported by its largest convertible bondholder representing approx. 32% of total bonds outstanding**
- **Santhera will call bondholder meeting for March 8, 2021, followed by Extraordinary General Meeting in March 2021**

Pratteln, Switzerland, February 16, 2021 – Santhera Pharmaceuticals (SIX: SANN) provides a corporate and pipeline update with near-term outlook and announces a proposed strengthening of its capital structure.

"The year 2020 was a challenging year for the Company, but I am delighted that Santhera has emerged in an operationally strong position with a promising future ahead. We have realigned the organization on delivering our promising drug candidate vamorolone to patients, having acquired full rights from ReveraGen and Idorsia in September 2020. Clinical studies with vamorolone have consistently delivered positive results and the pivotal VISION-DMD study is progressing with a topline 6-month data readout expected in the next quarter. With the potential for vamorolone to emerge as a foundational therapy for patients with DMD, regardless of the genetic mutation, regulatory activities are ongoing leading up to an FDA submission which we expect to be subject to priority review," said **Dario Eklund, CEO of Santhera**. "To satisfy the Company's immediate capital needs, we have secured additional financing from Highbridge. In addition, we propose a restructuring of the existing convertible bond which requires the consent of both bond- and shareholders and we are confident that we can count on their continued support. We see this strengthening of Santhera's capital structure as the best way to secure the Company's operations past the 6-month VISION-DMD data readout, after which, if positive, we will seek additional financing to fuel our future growth plans."

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Corporate/financial update and proposed capital restructuring

Update on corporate restructuring

The Company implemented an organizational restructuring to reduce costs and to focus on vamorolone as a consequence of the termination of the Puldysa program announced in October 2020. The workforce reduction and operating cost cutting programs are continuing as planned and scheduled to be completed by the end of March 2021. The result is a lower cost base and a streamlined and experienced organization, focused on progressing the development of vamorolone towards commercialization. The know-how of the core team in the development of DMD drug candidates, with extensive regulatory experience with the EMA and FDA, strong relationships with key clinical experts and the patient community, will be leveraged in order to bring vamorolone to patients.

Preliminary unaudited key financial figures 2020

Key consolidated financial results on a preliminary unaudited basis show for the year ended December 31, 2020, net sales of CHF 15.0 million, operating expenses of CHF 62.0 million, a net loss of CHF 71.2 million (2019: CHF 19.0 million) and, as of December 31, 2020, net cash of CHF 12.4 million and a net equity deficit of CHF 9.7 million.

This financial status has been prepared solely for financing purposes and can be viewed here; the Company expects to publish its 2020 Annual Report, with audited financial statements, on April 27, 2021.

Funding review, upsized financing from Highbridge proposed to satisfy immediate liquidity needs, and convertible bond restructuring

As of February 15, 2021, and taking into account the interest payment on the convertible bond scheduled for February 17, 2021, freely available cash and cash equivalents are projected to amount to CHF 6.9 million (unaudited). Management forecasts Santhera's operational cash burn rate to be around CHF 2.5 million per month over the coming few months.

Throughout 2020, under the leadership of the newly appointed CEO and CFO, Santhera succeeded in securing additional financings which extended cash reach into 2021. In addition to this already implemented funding, the Company has recently arranged a pathway to maintain a considerable liquidity buffer and further strengthen its capital structure. These actions combined would provide the Company with adequate liquidity runway to reach its next value-inflection point (expected in Q2-2021), and increase the likelihood of obtaining additional financing in future.

Santhera's existing investor Highbridge Tactical Credit Master Fund, L.P. (a fund managed by Highbridge Capital Management LLC, “Highbridge”) has already committed to increasing its existing financing arrangement with Santhera to provide up to CHF 18 million in senior secured notes exchangeable by Highbridge (CHF 6 million of which was previously committed), which will be available in tranches and subject to certain drawdown conditions. The maturity of such exchangeable notes has been extended to July 2022 and in consideration for this commitment and amendment, Highbridge will receive a fee in the

form of five-year warrants (options) for Santhera shares priced at a small premium to a reference share price determined ahead of the closing of the bond restructuring (see below).

In addition, Santhera proposes a restructuring of Santhera's existing CHF 60 million Senior Unsecured Convertible Bonds (Bonds, [1]), which Highbridge as the largest Bond investor with approx. 32% of the outstanding principal amount has agreed to support.

On February 17, 2021, the Company plans to publish an invitation to a bondholders' meeting. This meeting is scheduled to be held on March 8, 2021. Santhera proposes to the bondholders to (i) convert 32.5% of the principal value of each Bond (CHF 19.5 million in aggregate) into shares at the current conversion price of CHF 64.80 per share and (ii) modify the terms of the remaining 67.5% of principal value of each Bond (CHF 40.5 million in aggregate) as summarized below (the binding terms of the amendments are set out in the invitation to the bondholders' meeting):

1) reset the conversion price at 115% of the lower of (i) the VWAP of one share on February 15, 2021, and (ii) the average of the daily VWAP of one share during the five consecutive trading days immediately preceding the closing of the bond restructuring, but in all events not less than CHF 2.50, with a corresponding amendment of the formula applicable in a change of control;
2) extend the maturity date to August 17, 2024;
3) increase the interest rate to 7.50% per annum (currently: 5.00%) as after February 17, 2021, which would keep the absolute amount of interest payable on each payment date roughly the same;
4) introduce a new possibility of Santhera to pay interest in shares of Santhera, applying a discount of 10% to the then-prevailing market price of the shares;
5) interest make-whole: if a bondholder converts a Bond, Santhera will also pay the accrued interest up to the conversion date as well as the interest for the 36 months following the conversion date (or up to the maturity date, if shorter);
6) lowering of the threshold for Santhera's right to redeem the Bonds early to 150% of the conversion price (currently: 160%); and
7) increase bondholders’ rights under the Events of Default.

The proposed bondholder resolution requires a majority of two-thirds of all Bonds outstanding.

Santhera plans to call an extraordinary general meeting of shareholders, expected to be held in mid to end of March, 2021, where the Board will propose to shareholders, among other items, the authorization and issuance of the shares required to implement the bond restructuring and the upsized Highbridge financing. Such authorization will require a majority of two-thirds of all shareholders present at the shareholder meeting.

To assist in the assessment of strategic, balance sheet and liquidity options, the Company has retained Stifel Nicolaus Europe Limited as sole financial advisor. Together with its financial advisor, Santhera has determined that the proposed restructuring of the Bonds is required to enable Santhera to raise

additional financing, if the pivotal VISION-DMD readout expected for Q2-2021 is positive, and is therefore also crucial to preserve Santhera as a going concern until after such subsequent financing.

Pipeline and business development update

Vamorolone – the cornerstone of Santhera's pipeline

Santhera's activities are primarily focused on advancing vamorolone, a highly promising drug candidate for the treatment of patients with DMD. Santhera, together with its licensor ReveraGen, is currently studying vamorolone for early stage DMD patients requiring an anti-inflammatory, muscle strengthening treatment with a favorable tolerability profile to make it suitable for longer term administration and improving quality of life. Vamorolone binds to the same receptor as corticosteroids but modifies its downstream activity. This novel mode of action is believed to dissociate efficacy from typical steroid safety concerns [2-5]. In the currently completed studies, a total of 48 patients have received various doses of vamorolone; of which 41 patients have been treated and evaluated for a period of 2.5 years. Aggregate clinical data from these open label studies in DMD published to date showed sustained efficacy and clinical improvement with vamorolone across multiple endpoints coupled with a reduction of certain corticoid-specific side effects [6]. The Company believes vamorolone has the potential to become a foundational therapy in DMD for patients irrespective of the underlying gene mutation and a promising alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD.

In September 2020, Santhera secured full worldwide rights for vamorolone in all indications through agreements with Idorsia and ReveraGen, and assumed overall responsibility for the development program towards approval and commercialization. Based on the encouraging data available from previous studies, the Company is confident that the ongoing fully recruited pivotal Phase 2b VISION-DMD study [7-9], which enrolled 121 patients and compares vamorolone to a corticosteroid and placebo, will confirm previous efficacy and safety findings. A defined regulatory path for vamorolone has been pre-agreed in discussions with the US Food and Drug Administration (FDA) and on this basis Santhera is hopeful that a positive topline readout expected in Q2-2021 will meet the clinical requirements for a US new drug application (NDA) and approval. Vamorolone has been granted fast track status and rare pediatric disease designation, a requirement for a Priority Review Voucher, with the FDA.

The assembly of the technical module of the NDA (Common Technical Document, CTD) will require additional technical work to be performed by Santhera since bioequivalence between the licensors' formulation used in clinical trials and the formulation intended for upscaling and commercialization has not yet been demonstrated. This involves validation work with the existing clinical formulation to enable initial commercialization and is expected to result in a delay of NDA submission by approximately one quarter to Q1-2022 but, at the same time, will also allow for inclusion of 12-month data from the VISION-DMD trial for which a topline readout is expected in Q4-2021. In parallel, Santhera is initiating development of a next-generation follow-on formulation aiming at an improved scalability, cost reduction and potential additional intellectual property.

In Europe, discussions with the European Medicines Agency (EMA) indicated that the regulators will accept positive 12-month data from the VISION-DMD trial as a basis for a regulatory filing anticipated by mid-2022.

Further pipeline update and business development opportunities

Aside from vamorolone, Santhera continues to advance its clinical-stage candidate lonodelestat, a potent and selective peptide inhibitor of human neutrophil elastase (hNE), developed in cystic fibrosis. Data readout of the double-blind, placebo-controlled dose escalation Phase 1b study in patients with cystic fibrosis, to investigate safety, tolerability, pharmacokinetics and pharmacodynamics of orally inhaled multiple doses of lonodelestat for up to four weeks, is expected in the coming weeks (clinicaltrials.gov identifier: NCT03748199). Based on data from this early clinical proof of concept trial, the Company plans to design the further clinical development program. Available data from previous studies already demonstrated that single dose inhalation of lonodelestat can lead to high drug concentrations within the lung, resulting in inhibition of hNE in sputum of patients, and more results from this important biomarker are expected in the Phase 1b study. Neutrophil elastase is an enzyme associated with lung tissue inflammation, leading to degradation of the lung tissue in cystic fibrosis and several other pulmonary diseases [10].

With regard to the Company's proactive portfolio management strategy as an additional source of future non-dilutive income streams, the clarity achieved in the ownership structure of vamorolone lends itself to increased partnering opportunities for further expansion across different geographies (outside USA and the EU) and additional non-DMD indications for the benefit of patients worldwide. Similarly, the Company is currently also seeking opportunities for outlicensing agreements for lonodelestat in pulmonary indications beyond cystic fibrosis.

Note

The *Invitation to the Bondholders' Meeting* can be viewed under www.santhera.com/investors-and-media/investor-toolbox/share-bondholder-meetings from February 17, 2021, 07:00 hrs.

The *Financial Status* with preliminary unaudited financial figures as of December 31, 2020, has been prepared exclusively in view of the upcoming bondholders' meeting of Santhera Pharmaceuticals Holding AG and can be accessed under www.santhera.com/investors-and-media/investor-toolbox/share-bondholder-meetings. The Company expects to publish its 2020 Annual Report, with audited financial statements, on April 27, 2021.

Corporate calendar

February 17, 2021	Publication of invitation to Bondholder Meeting
March 8, 2021	Bondholder Meeting
Mid to end March, 2021	Extraordinary General Meeting
April 27, 2021	Publication of annual results and Annual Report 2020
June 22, 2021	Annual General Meeting

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References:
[1] Key bond data, Link
[2] Heier CR at al. (2013). EMBO Mol Med 5: 1569–1585.
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[6] Smith E, et al. (2020). PLOS Medicine, Link
[7] Clinicaltrials.gov. Link
[8] Santhera press release, September 11, 2020, Link
[9] VBP15-004, https://vision-dmd.info/2b-trial-information
[10] Barth P. et al (2019). Journal of cystic fibrosis, Link

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.

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