Santhera Pharmaceuticals Holding AG
Hohenrainstrasse 24, 4133 Pratteln, Switzerland
Phone: +41 61 906 89 50 | Fax: +41 61 906 89 51
www.santhera.com

Santhera Implements Reorganization and Secures Financing to Advance Vamorolone to Pivotal Read-out in Duchenne Muscular Dystrophy

- **Pivotal readout for vamorolone in Duchenne muscular dystrophy (DMD) expected in the second quarter of 2021**
- **Organizational restructuring to reduce workforce by 50%; annualized cost reductions of CHF 10 million**
- **Cost reductions combined with Highbridge facility amendment position Santhera for the readout of the VISION-DMD study, Santhera's next key value inflection point**
- **Partnering opportunities under evaluation for vamorolone and lonodelestat in non-core indications and geographies to enable proactive portfolio management**
- **Kristina Sjöblom Nygren, Chief Medical Officer and Head of Development, to leave Company by end 2020**

Pratteln, Switzerland, November 2, 2020 – Santhera Pharmaceuticals (SIX: SANN) provides a corporate update following implementation of an organizational restructuring and its move to a focus on vamorolone, a first-in-class dissociative steroid with a novel mode of action, to which the Company recently obtained global rights in all indications.

Vamorolone, the only dissociative steroid in development, is currently in a pivotal Phase 2b study in DMD patients with a 6-month readout expected in the second quarter of 2021. If successful, the results could lead to an NDA submission with the FDA in the fourth quarter of 2021 which is subject to a fast track review. Santhera currently anticipates to be first to market with a dissociative steroid in the US in 2022.

The refocusing on vamorolone, following discontinuation of Puldysa, necessitates a realignment of the organization to bring it in line with its new priorities and an extension of cash reach to advance the pipeline, above all vamorolone. Concluded amendments of the existing financing agreements with certain funds managed by Highbridge Capital Management, LLC (Highbridge) are to provide up to CHF 15 million, subject to certain conditions, of additional financing. In combination with the restructuring of the organization, available cash and additional measures, this is expected to extend Santhera's cash reach to its next major value enhancing inflection point in the second quarter 2021.

At the Executive Management level, Santhera will see a change with the departure of Kristina Sjöblom Nygren, Chief Medical Officer and Head of Development, for family reasons and to pursue other opportunities. The search for a successor to secure a seamless transition has been initiated.

"Moving forward as a Company with a focus on vamorolone, upon acquisition of global rights in all indications through agreements with Idorsia and ReveraGen and the termination of the Puldysa program, has forced us to take difficult decisions but at the same time has opened up new opportunities," said **Dario Eklund, Chief Executive Officer of Santhera**. "We are confident that the restructuring, internal cost reduction and the amended agreement with Highbridge will allow us to advance operations to the

topline readout in the second quarter of 2021 and will help us bring vamorolone to patients according to plan. Regretfully, we have to let go of many of our valued colleagues and I thank all of them for their dedication and efforts to do the very best for patients. In particular and on behalf of the entire management team, I would like to thank Kristina, who has played an important role in shaping and executing our clinical development strategy, for her valuable contributions to our Company. We wish her and others leaving all the best for their future. Looking forward, we are pleased to be able to count on an excellent core team committed to advancing our drug candidates and the continued support of investors recognizing the value of our pipeline products, above all vamorolone which would, if approved, be the first dissociative steroid to enter the US market."

Reorganization to realize cost reduction and advance vamorolone towards approval and market entry
The adjustment of Santhera's business activities is driven by the focus on vamorolone and the US, which is expected to be the first market for this drug candidate, and the need to extend cash reach to Santhera's next major value enhancing inflection point in the second quarter of 2021. Santhera is reducing its workforce by more than 50 positions to 47 full-time equivalent (FTE) employees. This restructuring will result in one-time costs of approximately CHF 3 million against recurring annual cost reductions of CHF 10 million. In addition, external development, marketing and other operating costs will decrease significantly as activities related to the terminated Puldysa program are closed out.

Furthermore, the Company will incur a one-time non-cash expense of CHF 9 million resulting from an impairment of inventory and other assets related to Puldysa as well as the reduction in infrastructure use arising from the organizational restructuring. Following the impairment, annualized amortization costs will be reduced by approximately CHF 1 million.

Cash runway sufficient to reach next inflection point and continue operations as planned
The existing financing with Highbridge has been amended to provide up to CHF 15 million in senior secured notes exchangeable by Highbridge, with CHF 5 million available immediately and the balance subject to certain conditions. These amendments replace Highbridge's existing unused financing commitments and are required to allow further drawdown following the termination of the Puldysa program. The Company's cash balances of CHF 10 million (as of October 31, 2020), together with the amended Highbridge facility, other financing and cost reduction initiatives, are expected to provide sufficient funding to reach the next major value enhancing inflection point, namely the 6-month topline readout of the VISION-DMD study with vamorolone.

In addition to balance sheet related cash inflows, the Company is entitled to staggered milestone payments of up to EUR 49 million, if and when license holder Chiesi Group meets certain sales thresholds for Raxone, and expects additional income from a 10% share of the Priority Review Voucher which may be granted to upon approval of vamorolone for DMD in the US.

Santhera's strategic near- and mid-term priorities
In summary, the near-term focus of Santhera will be on advancing its late-stage clinical drug candidates vamorolone and lonodelestat and securing sufficient funding to support operations beyond the second quarter of 2021. The Company expects a positive 6-month readout of the vamorolone VISION-DMD trial and a restructuring of the convertible bond to provide the basis to source additional capital aimed at

meeting future needs. For the mid-term, the Company is pursuing proactive portfolio management strategy through outlicensing agreements for both vamorolone (in non-DMD indications and geographies outside the US and Europe) and lonodelestat as an additional source of future non-dilutive income streams.

About vamorolone and the path forward

Santhera recently announced the completion of a license assignment from Idorsia (SIX: IDIA) and the early exercise of a licensing option to vamorolone with ReveraGen BioPharma, Inc (US: private). ReveraGen, together with Santhera, is developing 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. Vamorolone is a first-in-class drug candidate that binds to the same receptor as corticosteroids but modifies its downstream activity [1-4]. 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 concerns. Recently published data from open-label studies (VBP15-003 [5, 6] and VBP15-LTE [7, 8]) 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 corticosteroid-treated external control patients. Additionally, 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 [8]. On this basis, 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 6-month topline data from the fully enrolled pivotal VISION-DMD (VBP15-004 [9]) study are expected in the second quarter of 2021, based on which a regulatory submission to the FDA is planned for the fourth quarter of 2021. Subject to FDA approval, Santhera anticipates to be the first to market with a dissociative steroid in the US in 2022. The European regulatory authorities require 12-month treatment data and a filing with the EMA could occur in the first half of 2022 followed by launch approximately one year later. The Company 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 combined.

In parallel to the DMD program, Santhera is pursuing 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 has been obtained in in vitro and in vivo models for asthma, multiple sclerosis, inflammatory bowel disease, rheumatoid arthritis, dysferlin muscular dystrophy, critical illness muscle disease, and brain tumor [10]. In some of these diseases, the prescription of standard glucocorticoids is limited due to detrimental side-effects.

Vamorolone has been granted Orphan Drug status in the US and in Europe, has received Fast Track and Rare Pediatric Disease designations by the US FDA and obtained Promising Innovative Medicine (PIM) status from the UK MHRA.

References:

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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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