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Santhera Reports Net Revenues 2019 and Highlights Pipeline Progress

Pratteln, Switzerland, January 27, 2020 – Santhera Pharmaceuticals (SIX: SANN) generated net revenues of CHF 27.9 million in 2019 from sales of Raxone® for the treatment of Leber's hereditary optic neuropathy (LHON), which was out-licensed to Chiesi Group from August 2019. The Company made strong progress in advancing its lead neuromuscular compounds Puldysa® (idebenone) and vamorolone towards market entry and regulatory submission, respectively.

"We are pleased about Santhera's strong progress in 2019 and are excited about the prospects 2020 holds," said **Dario Eklund, CEO of Santhera**. "Our late-stage neuromuscular assets targeting the high value Duchenne muscular dystrophy (DMD) market are nearing key inflection points. With Puldysa (idebenone), we anticipate offering the first drug for the treatment of respiratory dysfunction for non-ambulant DMD patients who are not taking glucocorticoids. Vamorolone, a first-in-class anti-inflammatory drug candidate shown in studies to improve muscle and motor function with a favorable clinical safety profile, is being developed to replace glucocorticoids as standard of care in ambulant DMD patients. We will continue working with clinical experts, patient advocacy groups and regulators in order to bring these promising treatments to patients with currently few alternative treatment options as soon as possible."

2019 Turnover

- Net revenues CHF 27.9 million
- Gross income of CHF 49.3 million from Raxone out-licensing agreement
- Freely available liquid funds of CHF 31.4 million (December 31, 2019)

2019 full-year net revenues slightly above guidance

In 2019, Santhera reported net revenues of CHF 27.9 million (2018: CHF 31.7 million), slightly surpassing the Company's full-year guidance. This includes sales of Raxone in the approved indication Leber's hereditary optic neuropathy (LHON) in the first seven months of 2019. From August 2019, after the closing of the licensing transaction and the transfer of the Raxone-business to Chiesi Group, Santhera is commercializing Raxone for LHON in France in a transitional phase.

Upfront payment from Chiesi Group following closing of licensing agreement

In August 2019, Santhera recognized an initial gross income of CHF 49.3 million (EUR 44 million) from the licensing agreement with Chiesi Group. As previously announced per the agreement, 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 EUR 93 million.

As of December 31, 2019, freely available liquid funds (cash and cash equivalents) amounted to CHF 31.4 million (August 31, 2019: CHF 43.7 million). In addition, the Company held CHF 1.5 million of restricted cash designated for the interest payments related to the convertible bonds issued in 2017.

Pipeline and Regulatory Update

Anticipated near-term inflection points towards approval for both DMD pipeline candidates Puldysa (idebenone) and vamorolone are:

- Q2-2020: CHMP opinion on marketing authorization application for Puldysa in DMD in Europe
- Q4-2020: Launch of Puldysa in first European markets
- Q4-2020: Read-out of topline data of pivotal trial for vamorolone in DMD
- Q1-2021: Filing a New Drug Application for vamorolone in DMD in the US

Puldysa first launch in Europe in 2020 subject to positive opinion and approval from CHMP/EMA
The review of Santhera's application for conditional marketing authorization (CMA) for Puldysa in the treatment of respiratory dysfunction in patients with DMD who are not using glucocorticoids is ongoing and the Company expects an opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) around mid-2020. Subject to a positive opinion and subsequent EU approval, Santhera expects to launch Puldysa in the first European markets in late 2020.

SIDEROS DMD trial enrollment with idebenone nearing completion
Patient enrollment in Santhera's randomized, double-blind, placebo-controlled Phase 3 SIDEROS trial is expected to complete in Q2-2020 (clinicaltrials.gov: NCT02814019). With a study duration of 18 months, the last patient's last visit is scheduled for Q4-2021. If positive, the study data will allow for regulatory submissions supporting the use of Puldysa in all DMD patients experiencing respiratory decline, irrespective of their glucocorticoid use, in Europe and the US.

Vamorolone pivotal VISION-DMD study progressing as cornerstone for US/European regulatory filings
ReveraGen BioPharma is currently enrolling the Phase 2b VISION-DMD study with vamorolone, designed as a pivotal efficacy and safety trial (VBP15-004; clinicaltrials.gov: NCT03439670). Read-out of topline 6-month data from the randomized placebo-controlled treatment period is expected by Q4-2020 followed by an NDA submission in the US by Q1-2021. Under the agreements between the parties and upon exercising its option, Santhera would receive the sub-license to ReveraGen's vamorolone from Idorsia Ltd (SIX: IDIA) for all indications and all countries worldwide except Japan and South Korea. The marketing authorization application in Europe will require inclusion of 12-month data expected for Q2-2021.

Neuromuscular franchise a top priority for 2020
Santhera's strategic priorities for 2020 are its neuromuscular franchise: Puldysa and vamorolone in DMD. For Puldysa, the focus is on the preparation for European market entry in DMD later in the year and the completion of enrollment into the SIDEROS trial to support planned regulatory submissions, particularly in the US. For vamorolone, the key milestone will be the VISION-DMD topline data readout, which if positive would allow for preparation of the NDA filing and pave the way for Santhera's option exercise.

In parallel, the Company is advancing its clinical stage candidate POL6014 for cystic fibrosis and is evaluating further diversification of its platform type pipeline products, including development of additional indications in collaboration with partners.

Corporate Calendar

March 24, 2020 – Publication of the Annual Report 2019 (07h00 CET)
April 22, 2020 – Annual Shareholders' 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 is building a Duchenne muscular dystrophy (DMD) product portfolio to treat patients irrespective of causative mutations, disease stage or age. 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 anti-inflammatory drug candidate with novel mode of action, 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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