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Santhera Reports Preliminary Key Financial Figures for 2017 and Provides Corporate Update

Liestal, Switzerland, January 29, 2018 – Santhera Pharmaceuticals (SIX: SANN) announces preliminary, unaudited key financial figures for 2017. The Company reports an increase of 21% year-on-year in net revenues to CHF 22.9 million (2016: CHF 19.0 million) from sales of its lead product Raxone® for the treatment of Leber's hereditary optic neuropathy (LHON). Freely available liquid funds by year-end amounted to CHF 58.2 million (December 31, 2016: CHF 49.8 million). Following the recent negative opinion by the Committee for Medicinal Products for Human Use (CHMP) on its Marketing Authorization Application (MAA) for Raxone® in Duchenne muscular dystrophy (DMD), the Company will work with regulators and clinical experts to prepare a refiling as soon as possible. Other development projects progressed according to plan.

Financial and Commercial Highlights

- In 2017, Santhera reported net revenues from product sales of Raxone for LHON of CHF 22.9 million which corresponds to a growth of 21% year-on-year (2016: CHF 19.0 million). The roll-out of Raxone in the approved indication is progressing as planned and the product is currently sold in 20 European countries.
- By end of 2017, full reimbursement for Raxone in LHON was achieved for 8 European countries. In an additional 12 European countries, Raxone availability is currently governed by special reimbursement schemes.
- Commercial operations in the regional country clusters in Europe were expanded to support marketing of Raxone for LHON. In February, US operations were established in the Boston metropolitan area. The US team is currently focused on expanding relationships with patient advocacy groups and clinicians, supporting ongoing studies in the US, assembling a NDA filing for Raxone in DMD and preparing for market entry.
- In February 2017, Santhera successfully placed CHF 60 million senior unsecured convertible bonds due 2022. These funds are being primarily used for the commercialization of Raxone in the currently approved indication LHON, for investment into ongoing and further clinical trials with Raxone in DMD to facilitate regulatory filings, to advance the pipeline and for other corporate and business development purposes.
- As of December 31, 2017, freely available liquid funds (cash and cash equivalents and short-term financial assets) amounted to CHF 58.2 million (December 31, 2016: CHF 49.8 million). In addition, the Company reported CHF 7.5 million of restricted cash designated for the interest payments related to the convertible bonds during the first three years.
- The Company had 6,288,555 shares outstanding as of December 31, 2017.

"We are pleased about Santhera's strong commercial progress in 2017. At the same time, we are disappointed about the regulatory decision concerning the approval of Raxone in DMD," commented **Thomas Meier**, PhD, CEO of Santhera. "Our priorities for 2018 are clear: in the interest of patients and convinced of the treatment benefits of Raxone in DMD, we will work with clinical experts, patient advocacy groups and regulators to prepare for a refiling to enable treatment of patients with abnormal respiratory function and not taking glucocorticoids. In parallel, we will push ahead with the commercialization of Raxone in LHON and the advancement of our development pipeline."

Pipeline and Regulatory Matters and Outlook

- On January 26, 2018, Santhera announced that the European Medicines Agency's CHMP had maintained its negative opinion on the Type II extension application for Raxone® (idebenone) in DMD following a re-examination procedure. The CHMP concluded that an approval for Raxone in DMD applied as Type II variation of the existing marketing authorization cannot be granted at the present time based on the currently existing evidence base. Santhera remains fully committed to addressing this unmet need and is convinced of the treatment benefits of Raxone in DMD. The Company intends to strengthen the clinical data package for Raxone in preparation of a refiling of a Marketing Authorization Application (MAA) in Europe. In light of the CHMP's opinion, Santhera has withdrawn the corresponding regulatory application in Switzerland, with the intention to refile at a later stage.
- In June 2017, as the first drug for DMD, Raxone was granted a positive scientific opinion by the UK's Medicines and Healthcare products Regulatory Agency (MHRA) through the Early Access to Medicines Scheme (EAMS). This allows patients with DMD and respiratory function decline, who are not taking glucocorticoids and meet the criteria defined under this scheme, to gain access to Raxone already prior to marketing approval. The Company will seek consultations with MHRA concerning the continuation of the program.
- Santhera's randomized, double-blind, placebo-controlled Phase III trial (SIDEROS) designed to assess the efficacy of Raxone in delaying the loss of respiratory function in patients with DMD receiving concomitant glucocorticoid therapy is enrolling patients. The trial is currently recruiting patients in 56 centers in Europe and the US. The study duration is 18 months and completion of the trial is expected in H2 2020. If successful, this study will provide data that support use of Raxone in all DMD patients experiencing respiratory decline irrespective of their glucocorticoid use.
- In December 2017, Santhera announced the launch of an educational disease awareness campaign for the DMD community in the U.S. The "Take a Breath DMD" campaign, TakeABreathDMD.com, underscores the importance of respiratory care and also helps people living with DMD and their families receive information to help manage respiratory complications, including information about breathing, coughing and pulmonary care.
- The Phase I/II trial (IPPoMS) evaluating the safety and effectiveness of using Raxone to treat primary progressive multiple sclerosis (PPMS) has been completed. Top line study results of the trial which was carried out in collaboration with the U.S. National Institute of Neurological Disorders and Stroke (NINDS) are expected to be announced in Q1 2018.
- The Phase I trial (CALLISTO) evaluating the safety and tolerability of omigapil in pediatric and adolescent patients with congenital muscular dystrophy (CMD) was also conducted in collaboration with NINDS. The study has been completed and the announcement of top line results is planned for early Q2 2018. Omigapil has a Fast Track Designation and a grant from the FDA's Office of Orphan Products Development.

Guidance

- For 2017, Santhera anticipates a net result of CHF -50 to -55 million.
- For 2018, the Company expects net sales of Raxone for the currently approved indication LHON to reach CHF 28 to 30 million.

Conference call:

A conference call with Thomas Meier, PhD, CEO of Santhera, to discuss the results will be held today, January 29, 2018, at 14:00 hrs CET. Dial-in participants are invited to call one of the following numbers about 10 minutes before the conference call is due to start.

+41 58 310 50 00 (Europe)
+44 207 107 0613 (UK)
+1 631 570 5613 (USA)

Upcoming corporate events:

- Publication of the Annual Report 2017 on March 20, 2018, at 07:00 hrs CET
- Annual Shareholders' Meeting on April 12, 2018

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative pharmaceutical products for the treatment of orphan mitochondrial and neuromuscular diseases. Santhera's lead product Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON). Santhera is currently conducting the Phase III SIDEROS trial with Raxone® in patients with Duchenne muscular dystrophy (DMD) in respiratory function decline. In collaboration with the U.S. National Institute of Neurological Disorders and Stroke (NINDS) Santhera is developing Raxone® in a third indication, primary progressive multiple sclerosis (PPMS), and another product – omigapil – for congenital muscular dystrophy (CMD), both also areas of high unmet medical need. For further information, please visit the Company's website www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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