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Santhera Reports Preliminary Key Financial Figures for 2016 and Provides Corporate Update

Liestal, Switzerland, January 26, 2017 – Santhera Pharmaceuticals (SIX: SANN) announces preliminary, unaudited key financial figures for 2016. The Company reports net revenues of CHF 19.0 million (+340% year-on-year) from sales of its lead product Raxone® for the treatment of Leber's hereditary optic neuropathy (LHON). Santhera submitted Marketing Authorization Applications (MAA) for Raxone for the treatment of Duchenne muscular dystrophy (DMD) in the EU and Switzerland and made significant progress in all product development programs. Cash and cash equivalents by year-end amounted to CHF 49.8 million.

Commercial and Financial Highlights

- In 2016, the Company reported net revenues from product sales of CHF 19.0 million which is more than four-fold the net sales of the prior year (2015: CHF 4.3 million). Net sales for the second half of 2016 of CHF 11.8 million represent an increase of 64% compared to the first half of 2016 (CHF 7.2 million).
- Raxone was sold into 15 EU countries, with the majority of sales reached in France and Germany. Based on the number of packs sold, it is estimated that by year-end 2016 more than 280 LHON patients were receiving Raxone (year-end 2015: approx. 120).
- By end of 2016 full reimbursement for Raxone in LHON was achieved for Germany, Sweden, Norway, and Luxembourg. In several other countries, including France, Raxone availability is currently governed by special reimbursement schemes. The Company expects to reach full reimbursement in additional EU countries in 2017.
- By end of 2016 Santhera held CHF 49.8 million in cash and cash equivalents (end of 2015: CHF 76.9 million).
- For 2016, the Company expects a net result of CHF –33 to –38 million.
- The Company's outstanding shares amounted to 6'279'857 as of December 31, 2016.
- For 2017, Santhera expects net sales of Raxone for the currently approved indication to reach CHF 21 to 23 million.

Pipeline and Regulatory Highlights achieved in 2016

- Santhera submitted a MAA to the European Medicine Agency for Raxone for the treatment of DMD in patients with respiratory function decline and not taking concomitant glucocorticoids. Santhera expects an opinion from the Committee for Medicinal Products for Human Use (CHMP) on this second indication late in Q1 / early in Q2 2017.

- Santhera also submitted the corresponding MAA to the Swiss Agency for Therapeutic Products (Swissmedic).
- Santhera started a new randomized, double-blind, placebo-controlled Phase III trial (SIDEROS) designed to assess the efficacy of Raxone in delaying the loss of respiratory function in DMD patients receiving concomitant glucocorticoid therapy. The trial will be conducted in approximately 60 centers in Europe and in the US and has started to enroll patients. Treatment duration is 18 months and results of the SIDEROS trial are expected in H2 2019.
- The UK's Medicines and Healthcare Products Regulatory Agency (MHRA) designated Raxone as Promising Innovative Medicine (PIM) and as suitable candidate for entry into Step II of the Early Access to Medicines Scheme (EAMS).
- Santhera receives Fast Track Designation and a grant from the FDA's Office of Orphan Products Development for omigapil in congenital muscular dystrophy (CMD). The Phase I trial (CALLISTO) progresses as planned and the last patient's first visit is foreseen for Q2 2017 and results are currently planned to be available in H2 2017.
- The fully recruited Phase I/II trial (IPPoMS) with Raxone in primary progressive multiple sclerosis (PPMS) is conducted in collaboration with the US National Institute of Neurological Disorders and Stroke (NINDS). Completion of this trial is currently foreseen by late in 2017 with results to be announced end 2017 / early 2018.

"We are very excited about Santhera's strong progress last year, as witnessed by accelerating sales and regulatory submissions for DMD," commented **Thomas Meier**, PhD, CEO of Santhera. "While pushing ahead with the commercialization of Raxone in LHON, our main focus in 2017 will be on the preparation for market entry of Raxone as a therapy of DMD and the advancement of the SIDEROS trial."

Conference call:

An investor conference call with Thomas Meier, PhD, CEO of Santhera, will be held on January 26, 2017, at 13:00 hrs CET to discuss the results. Dial-in participants are invited to call on the following numbers about 10 minutes before the conference call is due to start.

+41 (0)58 310 50 00 (Europe)
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+1 (1)631 570 5613 (USA)

Upcoming corporate events:

- Release of the audited financial results for 2016 on March 7, 2017
- Annual Shareholders' Meeting on April 4, 2017

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 is authorized in the European Union, Norway, Iceland and Liechtenstein for the treatment of Leber's hereditary optic neuropathy (LHON). For Duchenne muscular dystrophy (DMD), the second indication for Raxone, Santhera has filed a Marketing Authorization Application (MAA) in the European Union and Switzerland. In collaboration with the US National Institute of Neurological Disorders and Stroke (NINDS) Santhera is developing Raxone in a third indication, primary progressive multiple sclerosis (PPMS), and omigapil for congenital muscular dystrophy (CMD), all 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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