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Santhera Announces Financial Results for the First Half-Year 2017 and Reports Commercial and Development Progress

Liestal, Switzerland, September 5, 2017 – Santhera Pharmaceuticals (SIX: SANN) reports first half-year results as of June 30, 2017:

- **1H 2017 sales of CHF 10.9 million, increase of 51% compared to 1H 2016**
- **Successful placement of CHF 60 million in convertible bonds**
- **Cash and short-term financial assets of CHF 78.0 million**
- **Continued roll-out of commercial launch of Raxone® for Leber's hereditary optic neuropathy (LHON)**
- **Positive Early Access to Medicines Scheme (EAMS) scientific opinion from the UK's Medicines and Healthcare products Regulatory Agency (MHRA) received for Raxone in Duchenne muscular dystrophy (DMD)**
- **European marketing authorization application (MAA) decision for Raxone in DMD from Committee for Medicinal Products for Human Use (CHMP) anticipated shortly**

Summarizing the half-year performance, **Thomas Meier**, PhD, Chief Executive Officer of Santhera, said: "We are on track to achieve our goals for 2017. On the commercial side, we have successfully advanced the European roll-out of Raxone for LHON and our commercial operations for our anticipated launch of Raxone in DMD are well underway. Recently, the UK's MHRA has granted Raxone a positive scientific opinion through the EAMS for patients with respiratory function decline not taking glucocorticoids in DMD. Another highlight was the successful placement of convertible bonds, which equipped us with adequate financial resources to implement our strategic and operational plans, and reflects the endorsement of the financial community in Santhera's future."

Company Highlights

- **Roll-out of Raxone for LHON well underway**
  Santhera's intention to make Raxone available for LHON patients across Europe are progressing well with new launches in several countries either through own subsidiaries or partnerships. Reimbursement was achieved under different models in several European markets. In May, the Scottish Medicines Consortium approved Raxone for restricted use in patients with LHON. By the end of the first half-year, Santhera sold Raxone in 17 European countries.

- **First positive EAMS Scientific Opinion from UK's MHRA in DMD**
  In June, the UK's MHRA granted Raxone a positive scientific opinion through the EAMS for patients with respiratory function decline not taking glucocorticoids in DMD. The MHRA decision allows patients with DMD, who meet criteria defined under this scheme, and who otherwise would not have access to such treatment options, to gain access to Raxone.

- **Review of marketing authorization application for DMD in Europe**
  The CHMP is currently assessing Santhera's extension application for Raxone in patients with DMD. An opinion from the CHMP is expected shortly.

- **Commercial operations strengthened to support LHON and prepare for launch in DMD**
  Commercial operations in the regional country clusters in Europe were increased to support marketing of Raxone for LHON and to prepare for a timely market entry of Raxone for DMD. In February, US operations were established in the Boston metropolitan area. The US team is currently focused on expanding relationships with patient advocacy groups, market preparation activities, and providing regulatory and medical affairs expertise. This expansion of geographic reach underscores Santhera's commitment to make Raxone available to patients worldwide.

- **SIDEROS trial with Raxone in DMD patients using glucocorticoids on track**
  Santhera's randomized, double-blind, placebo-controlled phase III SIDEROS study investigates the efficacy and safety of Raxone in DMD patients with declining respiratory function on any stable glucocorticoid treatment scheme. Currently, the last remaining of the targeted 62 study centers are being initiated. The study is expected to run until 2019 to support the use of Raxone in all patients with DMD experiencing respiratory decline irrespective of their glucocorticoid use status.

- **Israel approves Raxone for LHON**
  Post the period end, the Ministry of Health Israel approved Raxone for the treatment of visual impairment in adolescents and adult patients with LHON. This is the first approval for Raxone in LHON outside Europe.

Key Financials in the First Half-Year 2017

- **Strong uptake of Raxone sales**
  Net sales of Raxone amounted to CHF 10.9 million (1H 2016: CHF 7.2 million) which corresponds to a 51% increase compared to the same period of the year prior. Turnover was mainly driven by sales to LHON patients in Germany and France with additional markets contributing increasingly to growth. To date, Raxone is sold in 17 European countries.

- **Commercial and development activities reflected in increased expenses**
  Operating expenses in the first half-year were CHF 30.5 million (1H 2016: CHF 22.6 million). Advancing late stage clinical trials, as well as the follow-up and preparation of regulatory filings for DMD, led to higher development expenses of CHF 11.7 million (1H 2016: CHF 8.1 million). Marketing and sales expenses reached CHF 12.6 million (1H 2016: CHF 8.9 million) and general and administrative expenses (G&A) were CHF 6.1 million (1H 2016: CHF 5.5 million). This investment increase reflects the expansion of Santhera's operations, including set-up of the US subsidiary, the ongoing roll-out of Raxone for LHON across Europe and market entry preparations for Raxone in DMD. In summary, the operating loss in the first half of this year amounted to CHF 21.4 million (1H 2016: CHF –17.2 million) leading to a net result of CHF –22.7 million (1H 2016: CHF –18.0 million).

- **Successful placement of CHF 60 million convertible bonds**
  In February, Santhera successfully placed CHF 60 million senior unsecured convertible bonds with a 5-year maturity and a coupon of 5.00% per annum. Net proceeds from this placement will primarily be used to fund the commercialization of Raxone in the currently approved indication LHON, to prepare the market entry and commercial launch in the subsequent indications, and to further advance clinical development programs and for other corporate purposes. The additional funds significantly enhanced the Company's financial flexibility in executing its intended development and commercial plans.

- **Solid liquidity base allows for strategy implementation as planned**
  As of June 30, 2017, freely available liquid funds (cash and cash equivalents including short-term financial assets) amounted to CHF 78.0 million (December 31, 2016: CHF 49.8 million).

Revenue Guidance

Santhera reiterates its revenue outlook and currently expects net sales of Raxone for full year 2017 to reach CHF 21 to 23 million.

2017 Half-Year Financial Information

Please see www.santhera.com/investors-and-media/investor-toolbox/financial-reports for Santhera's 2017 interim condensed report and all reviewed consolidated financial statements.

Condensed interim consolidated income statement (reviewed, IFRS, for half-year ended June 30, in CHF thousands)	**1H 2017**	1H 2016
Net sales	**10,859**	7,210
Cost of goods sold (of which amortization of intangible assets: 2017 –1,519 / 2016 –1,519)	**–1,954**	–1,911
Development	**–11,703**	–8,101
Marketing and sales	**–12,622**	–8,949
General and administrative	**–6,113**	–5,479
Operating expenses	**–30,513**	–22,567
Operating result	**–21,366**	–17,207
Financial result	**–1,289**	85
Income taxes	**–57**	–849
Net result	**–22,712**	–17,971
Basic and diluted loss per share (in CHF)	**–3.62**	–2.87

Condensed interim consolidated balance sheet (IFRS, in CHF thousands)	**June 30, 2017 (reviewed)**	Dec. 31, 2016 (audited)
Cash and cash equivalents	**71,986**	49,815
Financial assets short-term	**5,984**	0
Noncurrent assets	**33,239**	28,442
Other current assets	**18,945**	12,535
Total assets	**130,154**	90,792
Equity	**55,199**	74,351
Noncurrent liabilities	**63,647**	6,183
Current liabilities	**11,308**	10,258
Total equity and liabilities	**130,154**	90,792

Condensed interim consolidated cash flow statement (reviewed, IFRS, in CHF thousands)	**2017**	2016
Operating cash flow for half-year ended June 30	**–19,431**	–13,338
Investing cash flow for half-year ended June 30	**–15,352**	–259
Financing cash flow for half-year ended June 30	**57,001**	336
Cash and cash equivalents at January 1	**49,815**	76,859
Cash and cash equivalents at June 30	**71,986**	63,564
Net change in cash and cash equivalents	**22,171**	–13,295

Share capital (number of shares with par value of CHF 1)	**June 30, 2017 (reviewed)**	Dec. 31, 2016 (audited)
Shares issued	**6,279,857**	6,279,857
Conditional capital for stock options	**700,000**	532,941
Conditional capital for convertible rights	**930,000**	650,000
Authorized capital	**1,500,000**	1,500,000

Call for Investors and Analysts

Santhera will host an investor call September 5, 2017 at 13:00 CET. Thomas Meier, PhD, CEO of Santhera, will discuss the half-year 2017 financial results and will provide an update on corporate developments.

Participants are invited to call the following numbers 10-15 minutes before scheduled call (no dial-in code required):
Europe: +41 (0)58 310 50 00
UK: +44 (0)203 059 58 62
US: +1 631 570 5613

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). For Duchenne muscular dystrophy (DMD), Santhera has filed a Marketing Authorization Application in the European Union and Switzerland for DMD patients with respiratory function decline who are not taking glucocorticoids. 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 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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