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A conference call will be held today at 13:00 CEST, 12:00 BST, 07:00 EDT. Details are at the end of this statement.

Santhera Announces Financial Results for the First Half-Year 2018 and Updates on Operational Progress and Growth Strategy

Pratteln, Switzerland, September 4, 2018 – Santhera Pharmaceuticals (SIX: SANN) reports first half-year results as of June 30, 2018, outlines the Company's vision and strategy as it moves through the second half of 2018, and positions itself for future growth.

Thomas Meier, PhD, Chief Executive Officer of Santhera, said: "Our vision is to be a leader in the development and commercialization of rare disease therapies for neuro-ophthalmology, neuromuscular and pulmonary indications. Our strategy to achieve this vision focuses on three distinct pillars: One, we continue to expand our commercial reach and grow sales of our revenue generating product Raxone® for the treatment of LHON. Turnover during the first half-year has been above expectation and we are on track to exceed our 2018 guidance. Two, we are progressing our pipeline assets towards regulatory approval in the EU and the U.S. and, with the inclusion of new data, intend to submit marketing authorization applications for idebenone in DMD in 2019. Three, we are pursuing an active in-licensing strategy for high quality, late-stage rare disease assets with a short time to market."

"We see multiple business development opportunities to leverage our existing development, regulatory and commercial capabilities and our recent in-licensing for POL6014 to treat cystic fibrosis is the first example of Santhera advancing this strategy. With this vision and strategy in mind, we believe Santhera is optimally positioned to create value with its existing and future product portfolio opportunities."

Financial highlights:

- 1H 2018 sales of CHF 16.0 million, increase of 48% compared to 1H 2017
- Operating expenses of CHF 39.9 million (1H 2017: CHF 30.5 million)
- Operating result of CHF –26.3 million (1H 2017: CHF –21.4 million) leading to a net result of CHF –27.4 million (1H 2017: CHF –22.7 million)
- Cash, cash equivalents and short-term financial assets of CHF 34.8 million (June 30, 2018)
- Full year sales guidance raised to CHF 30-32 million

Operational highlights:

- Acquisition of worldwide exclusive license to develop and commercialize clinical stage candidate POL6014 for cystic fibrosis (CF) and other pulmonary diseases
- Renewal of the Early Access to Medicines Scheme (EAMS) Scientific Opinion by UK's Medicines and Healthcare products Regulatory Agency (MHRA) for idebenone for patients with Duchenne muscular dystrophy (DMD) in the UK
- Launch of Expanded Access Program with idebenone for patients with DMD in the U.S.

- Submissions of regulatory dossiers for Raxone in Leber's hereditary optic neuropathy (LHON) in South Korea and Serbia
- Analysis of new data linking study findings with idebenone in DMD to clinically relevant patient benefits for inclusion in regulatory submissions in Europe and the U.S. (planned for 2019)
- Progress with clinical development candidates having successfully completed first clinical trial with omigapil in patients with congenital muscular dystrophy (CMD) and advanced preparations for multiple-ascending dose trial for POL6014 in CF

First half-year overview

- **Strong Raxone sales in 1H 2018**
  Net sales of Raxone in Europe amounted to CHF 16.0 million (1H 2017: CHF 10.9 million) which corresponds to a strong 48% increase year-on-year. Turnover was mainly driven by increased number of patients receiving the drug in existing markets and new launches in additional EU countries. Santhera's goal is to provide treatment to LHON patients worldwide and the Company has submitted a new drug application for LHON in South Korea, one of the major markets in Asia. A decision from the South Korean drug regulatory authorities who granted orphan drug designation for Raxone in LHON can be expected by summer 2019. At the end of the first half of 2018, Santhera was marketing Raxone in more than 20 countries.

- **Broadened product pipeline with licensing agreement**
  In February, Santhera completed the first step in its strategy to in-license pipeline strengthening, clinical stage product candidates in neuro-ophthalmology, neuromuscular and pulmonary diseases by entering into a license agreement with Polyphor for POL6014. Under the agreement, Santhera obtained the worldwide, exclusive rights to develop and commercialize POL6014, a clinical stage selective inhibitor of human neutrophil elastase with the potential to treat cystic fibrosis and other pulmonary diseases.

- **UK's MHRA renewed EAMS positive scientific opinion for Raxone in DMD**
  In June, the UK's MHRA renewed the EAMS scientific opinion for Raxone for a further year for patients with DMD in respiratory function decline who are not taking glucocorticoids. Inclusion in EAMS allows eligible patients with DMD, who meet criteria defined under this scheme, to gain free of charge access to Raxone in the UK.

- **Launch of U.S. Expanded Access Program with idebenone for patients with DMD**
  Santhera has successfully launched and enrolled the first patients in a U.S. Expanded Access Program (EAP), called BreatheDMD, with idebenone. Through the BreatheDMD program, eligible patients in the U.S. with DMD who are 10 years and older and in respiratory function decline can obtain access to investigational idebenone, at no cost, through a growing network of research centers across the U.S.

- **New supporting data to be included in submissions for marketing authorization applications for DMD in 2019**
  In July, Santhera announced results of a comparative analysis of the Phase III DELOS trial outcome with new data from natural history studies. This analysis showed that the treatment effect with idebenone observed in the DELOS trial can be linked to a delay in the initiation of assisted ventilation by three years, which is of high clinical relevance. In coming months, Santhera and its academic

partners will prepare for the publication of additional clinical data that demonstrate long-term efficacy of idebenone on respiratory function outcomes in patients with DMD, thereby supporting the positive data from the successful Phase III DELOS trial. The findings will be discussed with regulators in the coming months and will be included in the regulatory dossier in preparation of marketing authorization applications for idebenone in DMD in Europe and the U.S. in 2019.

- **Omigapil safe and well tolerated in patients with congenital muscular dystrophy (CMD)**
  The single-center interventional trial to establish the pharmacokinetic profile and to evaluate the safety and tolerability of omigapil in pediatric and adolescent patients with CMD was successfully completed. Santhera plans to seek advice on the clinical development program of omigapil by the TREAT-NMD Advisory Committee for Therapeutics (TACT).

- **Liquidity base allows for the continuation of the strategy as planned**
  As of the end of June 2018, Santhera had cash, cash equivalents and short-term financial assets of CHF 34.8 million (December 31, 2017: CHF 58.2 million). These funds will allow the Company to proceed with its clinical trial program and regulatory filings as foreseen.

Revenue Guidance

Santhera will continue to grow its international business, advance its pipeline programs and proceed business development initiatives to expand its late stage product portfolio. Based on its sales performance in the first six months of the current year and the positive outlook, the Company expects to exceed its guidance of CHF 28-30 million and anticipates reaching a higher turnover of CHF 30-32 million in 2018.

2018 Half-Year Financial Information

Please see www.santhera.com/investors-and-media/investor-toolbox/financial-reports for Santhera's 2018 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 2018**	1H 2017
Net sales	**16,027**	10,859
Cost of goods sold (of which amortization intangible assets: 2018 –1,519 / 2017 –1,519)	**–2,441**	–1,954
Development	**–18,854**	–11,703
Marketing and sales	**–12,921**	–12,622
General and administrative	**–8,051**	–6,113
Operating expenses	**–39,883**	–30,513
Operating result	**–26,297**	–21,366
Financial result	**–961**	–1,289
Income taxes	**–93**	–57
Net result	**–27,351**	–22,712
Basic and diluted loss per share (in CHF)	**–4.25**	–3.62

Condensed interim consolidated balance sheet (IFRS, in CHF thousands)	**June 30, 2018** **(reviewed)**	Dec. 31, 2017 (audited)
Cash and cash equivalents	**22,082**	45,195
Financial assets short-term	**12,742**	13,011
Other current assets	**21,394**	19,402
Noncurrent assets	**35,934**	32,172
Total assets	**92,152**	109,780
Equity	**15,177**	32,256
Noncurrent liabilities	**62,452**	64,278
Current liabilities	**14,523**	13,246
Total equity and liabilities	**92,512**	109,780

Condensed interim consolidated cash flow statement (reviewed, IFRS, in CHF thousands)	**2018**	2017
Operating cash flow for half-year ended June 30	**–22,154**	–19,431
Investing cash flow for half-year ended June 30	**137**	–15,352
Financing cash flow for half-year ended June 30	**–1,107**	57,001
Cash and cash equivalents at January 1	**45,195**	49,815
Cash and cash equivalents at June 30	**22,082**	71,986
Net change in cash and cash equivalents	**–23,113**	22,171

Share capital (number of shares with par value of CHF 1)	**June 30, 2018** **(reviewed)**	Dec. 31, 2017 (audited)
Shares issued	**6,527,479**	6,288,555
Conditional capital for stock options	**691,302**	691,302
Conditional capital for convertible rights	**930,000**	930,000
Authorized capital	**1,500,000**	1,500,000

Conference Call

Santhera will host a conference call on September 4, 2018 at 13:00 CEST / 12:00 BST / 07:00 EDT. Thomas Meier, PhD, CEO of Santhera, and Christoph Rentsch, CFO of Santhera, will discuss the half-year 2018 financial results and will provide an update on corporate developments.

Participants are invited to call one of the following numbers 10-15 minutes before the conference call starts (no dial-in code is required):

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

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein and Israel for the treatment of Leber's hereditary optic neuropathy (LHON) and is currently commercialized in more than 20 countries. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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