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Santhera Reports Solid Top-line Growth in 2017 and Reiterates its Strategy to Obtain Approval for Idebenone in Duchenne Muscular Dystrophy

Pratteln, Switzerland, March 20, 2018 – Santhera Pharmaceuticals (SIX: SANN) reports the Group's audited financial results for 2017. Net revenues from sales of lead product Raxone® (idebenone) increased by 21% compared to the previous year to CHF 22.9 million. The operating result of CHF –50.5 million reflects predominantly investments in several ongoing clinical development programs and preparations for regulatory submissions as well as commercial activities in Europe and the US. The net result amounted to CHF –51.5 million (2016: CHF –35.4 million), in line with the Company's guidance. With the in-licensing of a clinical stage drug candidate in February 2018, Santhera achieved the key objective of broadening its pipeline in rare diseases. Santhera will continue to review opportunities to expand its product pipeline for the treatment of rare neuro-ophthalmological, neuromuscular and pulmonary diseases. At the end of February 2018, the Company had cash, cash equivalents and short-term financial assets of CHF 50.9 million, allowing it to pursue its planned development and commercial activities.

"We made good operational progress in 2017 with continued commercial expansion, strong growth of sales for Raxone for Leber's hereditary optic neuropathy (LHON) and continuing progress in our rare disease pipeline," said **Thomas Meier**, PhD, CEO of Santhera. "Our strategy for future growth is twofold. First, we continue progressing idebenone through the regulatory pathway for the treatment of Duchenne muscular dystrophy (DMD) in Europe and the US as soon as possible. Whilst not obtaining approval in Europe was very disappointing, we remain convinced that the positive data from the Phase III DELOS trial form the basis of an approvable dossier. We are now focusing to collect supportive clinical data in preparation of a new Marketing Authorization Application in Europe."

"In addition, we are expanding our pipeline into three core therapeutic areas of rare neuro-ophthalmological, neuromuscular and pulmonary diseases. The first step of this strategy was completed with the in-licensing of the worldwide, exclusive rights to develop and commercialize POL6014, a clinical stage drug candidate for cystic fibrosis and other pulmonary diseases. For 2018, our priorities are clear: we will continue to drive growth in our commercial product, prepare the regulatory filings in DMD and continue to advance and grow our clinical development pipeline for rare neuro-ophthalmology, neuromuscular and pulmonary diseases."

Strong sales growth from Raxone for LHON

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.

The Company took the decision to reorganize the management of its commercial operations, in line with the strategic objective to organize its commercial operations in rare diseases on a global basis. Consequently, Giovanni Stropoli has stepped down from his role as Chief Commercial Officer Europe & ROW and Member of the Executive Management. We thank Giovanni Stropoli for his engagement and contribution to the successful launch of Raxone for LHON in Europe over the past 3 years.

Operating and net results reflect investment predominantly in clinical development and preparation for regulatory submissions as well as commercial operations

Late stage clinical studies such as the Phase III SIDEROS trial, post-authorization studies for LHON and the preparation of regulatory filings in Europe and the US led to higher development expenses of CHF 26.6 million (2016: CHF 17.7 million). Commercial operations in Europe were expanded to support marketing of Raxone for LHON. In February, US operations were established in the Boston metropolitan area to foster relationships with patient advocacy groups and clinicians, supporting ongoing studies in the US, preparing NDA filing for DMD and market entry. This resulted in higher marketing and sales expenses of CHF 28.5 million (2016: CHF 21.1 million) as well as general and administrative expenses of CHF 14.4 million (2016: CHF 9.8 million). In summary, total operating expenses were CHF 69.6 million (2016: CHF 48.6 million) and the operating result amounted to CHF –50.5 million (2016: CHF –33.1 million). For the full-year 2017, Santhera reported a net result of CHF –51.5 million (2016: CHF –35.4 million).

Well financed to implement business strategy as planned

In February 2017, Santhera successfully placed CHF 60 million senior unsecured convertible bonds due 2022. These funds are being primarily used for investment into clinical trials with idebenone in DMD to facilitate regulatory filings and for the commercialization of Raxone in the currently approved indication LHON and 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.

Broadened rare diseases product pipeline with focus on neuro-ophthalmological, neuromuscular and pulmonary diseases

In February 2018, Santhera entered into a license agreement with Polyphor Ltd. for POL6014, a clinical stage selective inhibitor of human neutrophil elastase with the potential to treat cystic fibrosis and other pulmonary diseases. Under the agreement, Santhera obtains the worldwide, exclusive rights to develop and commercialize POL6014 and its analogs. With the addition of this drug candidate, the Company broadened its clinical stage pipeline and extended the range of indications while maintaining a focus on providing therapies for rare diseases. Going forward, Santhera will be focusing its clinical development and commercialization efforts on orphan diseases from three therapeutic areas: neuro-ophthalmological, neuromuscular and pulmonary diseases.

Further information about Santhera's portfolio with clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases is available in the 2017 Annual Report on www.santhera.com.

Outlook and Guidance

Santhera will continue to grow its international business and advance its pipeline programs. Based on the sales performance in the first months of the current year the Company reiterates its guidance for net sales of Raxone in the currently approved indication LHON to reach CHF 28-30 million in 2018. The development priorities for 2018 are the clinical studies and data collection to support regulatory filings for idebenone in DMD and advancing the development of the other clinical stage candidates in the pipeline.

2017 Full-Year Financial Information

Santhera 2017 Annual Report see www.santhera.com/investors-and-media/investor-toolbox/financial-reports.

Condensed Consolidated Income Statement (IFRS, in CHF thousands)	**2017**	2016
Net sales	**22,943**	19,033
Cost of goods sold	**–4,104**	–3,883
Other operating income	**270**	361
Development	**–26,561**	–17,675
Marketing and sales	**–28,522**	–21,051
General and administrative	**–14,416**	–9,805
Other operating expenses	**–64**	–107
Operating expenses	**–69,563**	–48,638
Operating result	**–50,454**	–33,127
Financial result	**–821**	–67
Income taxes	**–257**	–2,221
Net result	**–51,532**	–35,415
Basic/diluted earnings/loss per share (in CHF)	**–8.22**	–5.65

Condensed Consolidated Balance Sheet (IFRS, in CHF thousands, as of December 31)	**2017**	2016
Noncurrent assets	**32,172**	28,442
Financial assets short-term	**13,011**	0
Cash and cash equivalents	**45,195**	49,815
Other current assets	**19,402**	12,535
Total assets	**109,780**	90,792
Equity	**32,256**	74,351
Noncurrent liabilities	**64,278**	6,183
Current liabilities	**13,246**	10,258
Total equity and liabilities	**109,780**	90,792

Condensed Consolidated Cash Flow Statement (IFRS, in CHF thousands)	**2017**	2016
Cash flow from operating activities	**–39,633**	–27,137
Cash flow from investing activities	**–22,239**	–459
Cash flow from financing activities	**57,108**	631
Cash and cash equivalents at January 1	**49,815**	76,859
Cash and cash equivalents at December 31	**45,195**	49,815
Net change in cash and cash equivalents	**–4,620**	–27,044

Share Capital (number of shares with par value of CHF 1, as of December 31)	**2017**	2016
Shares issued	**6,288,555**	6,279,857
Conditional capital for equity rights	**691,302**	532,941
Conditional capital for convertible rights	**930,000**	650,000
Authorized capital	**1,500,000**	1,500,000

Annual Report

The Santhera Annual Report 2017 is available for download on the Company's website at www.santhera.com/investors-and-media/investor-toolbox/financial-reports.

Upcoming Events

The Annual General Meeting of Santhera will be held on April 12, 2018, in Basel, Switzerland (shareholders will receive a separate invitation). The invitation can be downloaded from www.santhera.com/investors-and-media/investor-toolbox/shareholder-meetings.

About Santhera

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for orphan and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. The most advanced pipeline product, idebenone, is in clinical Phase III for the treatment of Duchenne muscular dystrophy (DMD). 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 currently commercialized in 20 countries. For further information, please visit www.santhera.com.

Raxone® is a trademark of Santhera Pharmaceuticals.

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