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Santhera Delivered Solid Performance in 2016 and Advances All Programs

Liestal, Switzerland, March 7, 2017 – Santhera Pharmaceuticals (SIX: SANN) reports the Group's audited financial results for 2016. The Company generated 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). The operating result amounted to CHF –33.1 million (2015 comparable: CHF –23.9 million) and the net result was CHF –35.4 million. Santhera submitted a Marketing Authorization Application (MAA) for Raxone for the treatment of Duchenne muscular dystrophy (DMD) in the EU and Switzerland, started the SIDEROS phase III trial in DMD and made significant progress in all other product development programs. Cash and cash equivalents by the end of February 2017 amounted to CHF 100.8 million.

"In the past year Santhera made excellent progress in all programs and 2016 marked our first full year as a commercial company," commented **Thomas Meier**, PhD, Chief Executive Officer of Santhera. "We are equally excited about Santhera's future. With our expanded geographic reach in Europe and the US, additional clinical trials and regulatory filings, we paved the way for further growth and value generation. Our strong financial position allows us to aggressively pursue our business strategy, to advance our products and establish Santhera as a leader in mitochondrial medicine."

Robust top-line growth driven by increasing Raxone sales

Net sales grew to CHF 19.0 million (2015: CHF 4.3 million) driven by strong sales of the lead product Raxone® for the treatment of Leber's hereditary optic neuropathy (LHON) in the EU.

Operating and net result reflects higher late stage development and market entry costs

Higher development expenses of CHF 17.7 million (2015: CHF 10.5 million) were attributable to costs associated with regulatory filings and the preparation and initiation of additional clinical trials. The ongoing commercial roll-out of Raxone across Europe, preparations for market entry in the second indication DMD and the build-up of US operations resulted in higher expenses for marketing and sales of CHF 21.1 million (2015: CHF 8.4 million) and general and administrative of CHF 9.8 million (2015: CHF 8.2 million). In summary, total operating expenses were CHF –48.6 million (2015 comparable: CHF –27.1 million) and the operating result amounted to CHF –33.1 million (2015 comparable: CHF –23.9 million). Comparable figures are provided to account for the extraordinary reversal of a previous impairment charge of CHF 27.1 million (included in operating expenses) following the approval of Raxone for LHON. Group net result in 2016 amounted to CHF –35.4 million (2015: CHF 5.9 million).

Strong cash position allows for implementation of strategy as planned

Cash-flow from operating activities amounted to CHF –27.1 million (2015: CHF –22.4 million). As of December 31, 2016, Santhera had cash and cash equivalents of CHF 49.8 million (2015: CHF 76.9 million). In February 2017, after the balance sheet date, Santhera successfully placed CHF 60 million senior unsecured convertible bonds due 2022, resulting in a cash position by end of February 2017 of CHF 100.8 million. These cash reserves provide the financial flexibility to support the Company's, development, regulatory and commercial projects as planned.

Santhera established international operations

2016 was marked by a strong internationalization of Santhera's business activities. In parallel to the commercial roll-out of Raxone, Santhera expanded its operations across Europe and, by the end of 2016, sold Raxone into 15 countries. In February 2017, Santhera established presence in the United States with its US-subsidiary Santhera Pharmaceuticals (USA), Inc. in the Boston metropolitan area, one of the main centers for pharmaceutical companies in North America. The US team currently intensifies relations to patient advocacy groups, prepares market access and provides regulatory and medical affairs expertise.

Strong progress in all development programs

In 2016, Santhera submitted Marketing Authorization Applications (MAA) for Raxone for the treatment of Duchenne muscular dystrophy (DMD) in the EU and Switzerland and significantly advanced all product development programs. Santhera also started the randomized, double-blind, placebo-controlled phase III trial (SIDEROS) to assess the efficacy of Raxone in delaying the loss of respiratory function in DMD patients receiving concomitant glucocorticoid therapy. Details on progress made with Raxone and the development programs in 2016 were previously announced (press release of January 26, 2017) and are described in the Annual Report 2016.

Outlook and Guidance

For the Company's Annual Shareholders' Meeting on April 4, 2017, the Board proposes Philipp Gutzwiller, Elmar Schnee, Patrick Vink, MD, and Thomas Meier, PhD, CEO of Santhera, for election as new members of the Board of Directors. Subject to his election, Thomas Meier will also be appointed Delegate of the Board. With these nominees, the Board will gain additional experienced senior executives to strengthen the strategic expertise in global pharmaceutical business growth. Jürg Ambühl, Board Member of Santhera since 2009, has decided not to stand for re-election. The Board and management thank Mr. Ambühl for his many valuable contributions and his strong commitment to the Company during these decisive years.

Santhera will continue to grow its international business. The Company expects 2017 net sales of Raxone in the currently approved indication alone to reach CHF 21 to 23 million. Besides focusing on commercialization and reimbursement of Raxone in LHON in Europe, additional priorities in 2017 will entail preparations for market entry and launch for Raxone in the second indication DMD, and advancement of all currently ongoing clinical trials.

2016 Full-Year Financial Information

Santhera 2016 Annual Report see www.santhera.com/investors-and-media/investor-toolbox/financial-reports.

Condensed Consolidated Income Statement (IFRS, in CHF thousands)	**2016**	2015
Net sales	**19,033**	4,321
Cost of goods sold	**–3,883**	–1,371
Other operating income	**361**	188
Development	**–17,675**	16,651
Marketing and sales	**–21,051**	–8,356
General and administrative	**–9,805**	–8,244
Other operating expenses	**–107**	–16
Operating expenses	**–48,638**	35
Operating result	**–33,127**	3,173
Financial result	**–67**	–239
Income taxes	**–2,221**	3,015
Net result	**–35,415**	5,949
Basic earnings/loss per share (in CHF)	**–5.65**	1.11
Diluted earnings/loss per share (in CHF)	**–5.65**	1.08

Condensed Consolidated Balance Sheet (IFRS, in CHF thousands, as of December 31)	**2016**	2015
Cash and cash equivalents	**49,815**	76,859
Noncurrent assets	**28,442**	33,208
Other current assets	**12,535**	7,085
Total assets	**90,792**	117,152
Equity	**74,351**	106,247
Noncurrent liabilities	**6,183**	3,957
Current liabilities	**10,258**	6,948
Total equity and liabilities	**90,792**	117,152

Condensed Consolidated Cash Flow Statement (IFRS, in CHF thousands)	**2016**	2015
Cash flow from operating activities	**–27,137**	–22,390
Cash and cash equivalents at January 1	**76,859**	17,435
Cash and cash equivalents at December 31	**49,815**	76,859
Net change in cash and cash equivalents	**–27,044**	59,424

Share Capital (number of shares with par value of CHF 1, as of December 31)	**2016**	2015
Shares issued	**6,279,857**	6,262,798
Conditional capital for stock options	**532,941**	401,694
Conditional capital for convertible rights	**650,000**	650,000
Authorized capital	**1,500,000**	910,000

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Annual Report

The Santhera Annual Report 2016 is available for download on the Company's website at www.santhera.com/investors-and-media/investor-toolbox/financial-reports.

Upcoming Events

The Annual Shareholders' Meeting of Santhera will be held on April 4, 2017, in Basel, Switzerland (shareholders will receive a separate invitation).

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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