Santhera Pharmaceuticals Holding AG
Hammerstrasse 49
4410 Liestal / Switzerland
Phone +41 61 906 89 50
Fax +41 61 906 89 51
www.santhera.com

Santhera Announces Financial Results for the First Half Year 2016 and Reports Solid Sales Growth

Company Starts to Build US Operations and Strengthens Board of Directors

Liestal, Switzerland, September 6, 2016 – Santhera Pharmaceuticals (SIX: SANN) announces its financial results for the first half year 2016 and reports solid sales growth. By end of the reporting period sales of Raxone® for Leber's hereditary optic neuropathy (LHON) had reached CHF 7.2 million, recorded primarily in Germany and France with an increasing sales contribution from additional mid-sized markets. Santhera has filed a Marketing Authorization Application (MAA) in Europe for Raxone for the treatment of Duchenne muscular dystrophy (DMD) in patients not taking glucocorticoids. The MAA was submitted as Type II variation of the existing marketing authorization for LHON and is currently under review by the Committee for Medicinal Products for Human Use (CHMP). Santhera will shortly start a randomized, double-blind, placebo-controlled phase III (SIDEROS) trial to assess the efficacy of Raxone in DMD patients receiving concomitant glucocorticoids. If successful, data from this trial will be used to support a label extension to include all DMD patients irrespective of their glucocorticoid use status. Santhera will also approach the US Food and Drug Administration (FDA) with the intent to re-engage in further discussions on the accelerated approval pathway for the glucocorticoid non-using patients, in whom clinically relevant benefit with Raxone has already been demonstrated.

Santhera also reports that it has started to build US operations under the leadership of Todd Bazemore, who was appointed as Chief Operating Officer of Santhera Pharmaceuticals (USA) Inc. and member of Santhera's Executive Management Team. In addition, the Company announces the nomination of Patrick Vink, MD to Santhera's Board of Directors. Patrick Vink is a senior life science executive with a track-record in building global pharmaceutical businesses.

Thomas Meier, PhD, Chief Executive Officer of Santhera, commented on the first six months of 2016: "The highlights in the first half year were the solid growth of Raxone sales for LHON in initial markets in Europe and the submission of our MAA for DMD. The sales uptake has been somewhat slower than originally expected due to the complex pricing and reimbursement processes in which we are currently engaged in several EU markets. As such decisions are expected in the coming months, we anticipate further growth of Raxone sales in the second half of 2016. Whilst we await the outcome of the ongoing review of our MAA for DMD, we are already actively preparing for market entry."

Building US Operations – Appointment of Todd Bazemore as Chief Operating Officer of Santhera Pharmaceuticals U.S. and Member of Santhera's Executive Management Team

Santhera is establishing US operations in the Greater Boston Area under the leadership of **Todd Bazemore** appointed today as Chief Operating Officer of Santhera Pharmaceuticals (USA) Inc. He joins Santhera as a biopharmaceutical executive with 22 years of experience in launching and building brands and has been instrumental in the success of a number of drugs across multiple therapeutic areas, spanning from ultra-rare diseases to large primary care conditions. Prior to joining Santhera Todd Bazemore served as EVP & Chief Commercial Officer at Dyax Corp. where he was responsible for global commercial strategy and oversaw all commercial functions. Dyax was acquired by Shire plc in January of this year. The US operations will initially be staffed to focus on regulatory and clinical operations support, medical affairs, patient advocacy liaison and commercial strategy.

"The US market is a key driver of Santhera's long-term valuation," commented **Thomas Meier**. "I am very pleased that we establish our US operations now as we plan to engage the FDA in further discussions about accelerated approval for Raxone in DMD. Todd's strong track record of strategic leadership, operational execution and launching and building brands will be highly valuable as we develop our commercial strategy for the US market. In this important role, Todd will join our Executive Management Team."

Extending Board of Directors

Santhera plans to extend its Board of Directors with the nomination of Patrick Vink, MD and senior life science executive with a track-record of successfully managing global businesses. As a former member of company executive teams, he adds extensive experience in interacting with key stakeholders like investors, the financial community and government officials both from European and US perspectives. In his most recent operational role he was Executive Vice President and Chief Operating Officer at Cubist Pharmaceuticals. Patrick Vink acts as Advisor to the Board of Directors with immediate effect and is designated for election as a new member of the Board of Directors at the Company's 2017 Annual Shareholder Meeting.

On the extension of the Board of Directors **Martin Gertsch**, Chairman of Santhera's Board of Directors commented: "I am very delighted about the nomination of Patrick Vink to our Board of Directors. Patrick's strong track record of growing global life science businesses and his strategic leadership nicely complements the expertise of the current members of the Board of Directors."

Key Financials in the First Half Year

- **Increasing sales for Raxone drove topline growth**
  In the first six months of 2016, Raxone generated net sales of CHF 7.2 million (1H 2015: CHF 1.5 million; 2H 2015: CHF 2.8 million), mainly driven by increased Raxone sales to LHON patients in Germany and France.

- **Intensified commercial and clinical activities increased operating expenses**
  Operating expenses in the first half-year were CHF 22.6 million (1H 2015: CHF 7.5 million). Preparation of regulatory filings for DMD in Europe and the US and the implementation of late

stage clinical trials led to higher development expenses of CHF 8.1 million (1H 2015: CHF 2.9 million). Marketing and sales expenses rose to CHF 8.9 million (1H 2015: CHF 1.5 million) and general and administrative expenses (G&A) to CHF 5.5 million (1H 2015: CHF 3.1 million). These increases reflect the expansion of Santhera's operations, especially the commercial activities, the ongoing roll-out of Raxone for LHON across Europe and market entry preparations for Raxone for DMD. In summary, the operating loss amounted to CHF 17.2 million (1H 2015: CHF –6.2 million) leading to a net result of CHF –18.0 million (1H 2015: CHF –6.4 million).

- **Sound financial basis to advance commercial and development strategies as planned**
  As of June 30, 2016, Santhera had cash and cash equivalents of CHF 63.6 million (December 31, 2015: CHF 76.9 million). Net change in cash and cash equivalents in the first half year of 2016 was CHF –13.3 million.

Company Highlights

- **Solid uptake of Raxone for LHON in Europe**
  By end of the reporting period Raxone sales were recorded primarily in Germany and France with an increasing sales contribution from additional mid-sized markets. Santhera expects reimbursement decisions by a number of European Authorities in the second half 2016 and early 2017.

- **Relevance of pulmonary benefits for patients with DMD reconfirmed at first "Duchenne Pulmonary Outcomes Workshop"**
  In April 2016, Santhera participated in the "Duchenne Pulmonary Outcomes Workshop," organized by Parent Project Muscular Dystrophy (PPMD), the leading U.S. advocacy organization working to end Duchenne. The workshop convened experts in the research and clinical care of DMD patients who examined current and future assessments of pulmonary function. Santhera presented data from its phase III DELOS trial, which demonstrated clinically relevant efficacy of Raxone (idebenone) in preserving respiratory function, a key objective for DMD therapy. Previously, a patient and caregiver survey conducted by PPMD clearly demonstrated that the DMD community highly values treatment options for pulmonary complications.

- **Marketing Authorization Application (MAA) filed in Europe for Raxone for DMD**
  In May 2016, Santhera submitted a MAA to the European Medicines Agency (EMA) for Raxone for the treatment of DMD in patients with respiratory function decline and not taking concomitant glucocorticoids. The new indication was submitted as Type II variation of the Company's existing marketing authorization for Raxone which was granted last year. Shortly thereafter, on June 21, the EMA validated Santhera's application thereby confirming that the submission is complete and the review process by the Committee for Medicinal Products for Human Use (CHMP) has begun.

- **Update on US filing strategy for DMD**
  In July 2016, Santhera reported that the FDA commented on the proposed subpart H approval pathway and requested that a second phase III trial be completed providing additional data to support NDA filing for Raxone in all DMD patients, irrespective of their glucocorticoid use status. The FDA confirmed that a positive outcome of the planned SIDEROS trial has the potential to provide the supplementary efficacy data to support NDA filing in all DMD patients whether they

use glucocorticoid or not. Santhera will work closely with the DMD patient community and clinical experts with the intent to engage the FDA in further discussions on an accelerated pathway to approval in the glucocorticoid non-using patients, in whom clinically relevant benefit has already been demonstrated.

- **Publication on bronchopulmonary benefits of Raxone in DMD in *Neuromuscular Disorders***
  In June, additional data from the pivotal phase III trial (DELOS) were published in *Neuromuscular Disorders*, the official journal of the World Muscle Society (McDonald et al., Neuromuscular Disorders 2016, 26: 473–480). These data show that DMD patients treated with Raxone have a reduced risk of bronchopulmonary complications including fewer hospitalizations caused by such complications and a reduced need for systemic antibiotic treatment compared to patients receiving placebo.

- **Received US fast-track designation for omigapil – CALLISTO study on track**
  In May, Santhera received Fast Track Designation from the FDA for omigapil for the treatment of congenital muscular dystrophy (CMD). Previously, omigapil was granted Orphan Drug Designation for CMD in both the EU and the US. Santhera, in collaboration with the US National Institutes of Health (NIH), is currently conducting a clinical phase I study (CALLISTO) with omigapil in CMD patients. CALLISTO assesses the pharmacokinetics, safety, and tolerability of omigapil in ambulatory and non-ambulatory children affected by CMD. On August 30, Santhera announced that the Office of Orphan Products Development (OOPD) at the FDA has granted Santhera an award of USD 246'000 in support of its ongoing CALLISTO trial.

- **SIDEROS trial with Raxone in DMD-patients using glucocorticoids to start imminently**
  The first patient is expected to be enrolled shortly in Santhera's randomized, double-blind, placebo-controlled phase III SIDEROS study. The trial is designed to confirm the efficacy of Raxone in patients currently taking glucocorticoids who are experiencing respiratory function decline, a patient population previously not enrolled in the positive phase III DELOS trial. If successful, this study will provide data to support the use of Raxone in all DMD patients experiencing respiratory decline irrespective of their glucocorticoid use status. Raxone for DMD was granted Orphan Drug Designation in the EU and the US and Fast Track Designation in the US.

Outlook and Guidance

The Marketing Authorization Application for Raxone in DMD is currently under review by the CHMP and Santhera expects a response from the regulatory authority in the first quarter 2017.

In July 2016, Santhera was advised by the FDA that the successful completion of the SIDEROS trial together with data from the previously successful phase III DELOS trial will provide the necessary data to support NDA filing for Raxone in all DMD patients irrespective of the glucocorticoid use status. In the interest of patients and due to the fact that the benefit of Raxone has already been demonstrated in the glucocorticoid non-using patients, Santhera will approach the FDA with the intent to re-engage in further discussions on an accelerated pathway to approval specifically for this patient population.

Santhera currently expects net sales of Raxone in 2016 to reach CHF 16 to 18 million.

2016 Half Year Financial Information

See www.santhera.com for Santhera's 2016 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 2016**	1H 2015 [1]
Net sales	**7,210**	1,455
Cost of goods sold (of which amortization of intangible assets: 2016 –1,519 / 2015 0)	**–1,911**	–159
Development	**–8,101**	–2,863
Marketing and sales	**–8,949**	–1,535
General and administrative	**–5,479**	–3,133
Operating expenses	**–22,567**	–7,535
Operating result	**–17,207**	–6,216
Financial result	**85**	–162
Income taxes	**–849**	–2
Net result	**–17,971**	–6,380
Basic and diluted loss per share (in CHF)	**–2.87**	–1.28

Condensed interim consolidated balance sheet (IFRS, in CHF thousands)	**June 30, 2016** **(reviewed)**	Dec. 31, 2015 (audited)
Cash and cash equivalents	**63,564**	76,859
Noncurrent assets	**31,015**	33,208
Other current assets	**10,301**	7,085
Total assets	**104,880**	117,152
Equity	**89,190**	106,247
Noncurrent liabilities	**5,601**	3,957
Current liabilities	**10,089**	6,948
Total equity and liabilities	**104,880**	117,152

Condensed interim consolidated cash flow statement (reviewed, IFRS, in CHF thousands)	**2016**	2015
Operating cash flow for half year ended June 30	**–13,338**	–7,167
Cash and cash equivalents at January 1	**76,859**	17,435
Cash and cash equivalents at June 30	**63,564**	10,476
Net change in cash and cash equivalents	**–13,295**	–6,959

Share capital (number of shares with par value of CHF 1)	**June 30, 2016** **(reviewed)**	Dec. 31, 2015 (audited)
Shares issued	**6,274,598**	6,262,798
Conditional capital for stock options	**538,200**	401,694
Conditional capital for convertible rights	**650,000**	650,000
Authorized capital	**1,500,000**	910,000

[1] Some amounts have been restated in comparison with the Interim Report 2015. Further details related to the nature of the corrections are outlined in the Annual Report 2015 (note 2 “Correction of errors”).

Call for Investors and Analysts

Santhera will host an Investor Call on September 6, 2016 at 5pm CET. Thomas Meier, CEO of Santhera, will discuss the Half Year 2016 Financial Results and will provide an update on corporate developments.

Participants can call the following numbers 10-15 minutes before Conference's schedule (no dial-in code needed):
+41 (0)58 310 50 00 (Europe)
+44 (0)203 059 58 62 (UK)
+1 (1)631 570 5613 (USA)

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. 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.

For further information, contact:

Thomas Meier, PhD, Chief Executive Officer
Phone +41 61 906 89 64
thomas.meier@santhera.com

Christoph Rentsch, Chief Financial Officer
Phone +41 61 906 89 65
christoph.rentsch@santhera.com

Daniel Piller, Head Communications
Phone +41 61 906 89 26
daniel.piller@santhera.com

US investor contact:
Hans Vitzthum, LifeSci Advisors, LLC
Phone +1 212 915 2568
hans@lifesciadvisors.com

US Public Relations contact:
Deanne Eagle, Planet Communications
Phone +1 917 837 5866
deanne@planetcommunications.nyc

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