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Santhera Reports 2014 Financial Results and Significant Progress in All Programs

Liestal, Switzerland, April 14, 2014 – Santhera Pharmaceuticals (SIX: SANN) announces the financial results for the full year 2014 and reports significant progress in all programs. The Company increased the income from sales of Raxone®, submitted an application for marketing authorization (MAA) in Leber's Hereditary Optic Neuropathy (LHON), reported the successful outcome of its Phase III trial in Duchenne Muscular Dystrophy (DMD), and secured adequate funds to advance all strategic projects and prepare for market entry.

Commenting on the results, **Thomas Meier**, PhD, CEO of Santhera, said: "We report excellent progress in all strategic programs for Raxone and have reached a number of important milestones. Among the many highlights in 2014, the outstanding events were the re-filing of the Marketing Authorization Application for Raxone to treat LHON and the positive results from our Phase III study in DMD. From a financial point of view, the increased income from sales of Raxone and two private placements in February and August 2014 substantially strengthened Santhera's cash position. The available cash ensures that adequate funds are available in support of ongoing preparations for a potential launch of Raxone for the treatment of LHON in Europe, and to advance other development and regulatory projects."

Financial Highlights:

- **Financing and income from Raxone sales increased cash position**
  As of December 31, 2014, Santhera had cash and cash equivalents of CHF 17.4 million (2013: CHF 5.0 million) which corresponds to a net year-on-year increase of CHF 12.4 million (2013: CHF –7.2 million). The Company realized an aggregate gross amount of CHF 15.7 million through private share placements, sale of treasury shares and sale of shares under the Standby Equity Distribution Agreement, which together with increasing income from product sales contributed to the strong cash position.

- **Top-line growth driven by increasing sales of Raxone**
  Mainly due to increasing top-line growth in the second half year, net sales 2014 climbed to CHF 2.6 million (2013: CHF 1.3 million). Increasing sales were generated with Raxone for LHON under the French temporary authorization for use (cATU) and with the international Named Patient Programs for patients with DMD and LHON.

- **Operating and net result at previous year's level**
  Development expenses increased to CHF 5.7 million (2013: CHF 4.7 million). Preparations for market entry contributed to higher general and administrative expenses of CHF 4.2 million (2013: CHF 3.1 million). Overall, the operating result of CHF –7.5 million was comparable to 2013 (CHF –7.3 million).

For the full-year 2014, Santhera reports a net result of CHF –7.5 million (2013: CHF –5.8 million including extraordinary financial income of CHF 1.5 million from the settlement of finance lease liabilities).

Product and Pipeline Highlights:

- **Received temporary approval in France for Raxone as first treatment for LHON**
  In January 2014, the French National Agency for Medicines and Health Products Safety granted a temporary approval (cATU) for Raxone to treat patients with LHON. This cATU was renewed for 2015 and allows patients in France to receive reimbursed treatment with Raxone before a marketing authorization is granted in the European Union.

- **Re-filed European MAA for Raxone in LHON – Regulatory review in progress**
  In May 2014, Santhera re-filed an MAA with efficacy data from the pivotal RHODOS study and additional clinical efficacy data from an Expanded Access Program. An opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency is expected in H1 this year. Subject to approval, Santhera plans to launch Raxone as the first product authorized in Europe for the treatment of LHON.

- **Reported positive results of the DELOS Phase III trial with Raxone/Catena in DMD**
  In May 2014, Santhera announced the positive outcome of the placebo-controlled Phase III trial with Raxone in DMD. The study met its primary endpoint and demonstrated that Raxone can delay the loss of respiratory function in patients not using glucocorticoid steroids. The preservation of respiratory function is considered of major clinical importance for Duchenne patients.

- **Initiated collaboration with Parent Project Muscular Dystrophy (PPMD)**
  In November 2014, Santhera announced a collaboration agreement with the US patient advocacy organization PPMD for a survey-based benefit/risk evaluation in DMD. The survey will focus specifically on patient and caregiver preferences regarding pulmonary therapies and will be used in support of the Company's NDA filing in the USA.

- **Continued collaborative Phase II trial with the US National Institutes of Health (NIH) on Raxone/Catena in primary progressive Multiple Sclerosis (ppMS)**
  Santhera continues its collaboration with the NIH in a double-blind, placebo-controlled Phase II clinical trial investigating the efficacy of Raxone/Catena in patients with ppMS, the compound's third indication. The trial, which combines a 1-year observational run-in phase followed by a 2-year randomized, placebo-controlled treatment period is now fully enrolled.

- **Initiated a clinical development program with omigapil in Congenital Muscular Dystrophies (CMD) with support from private-public partnership**
  In July 2014, Santhera announced a public-private partnership with the NIH, EndoStem, an EU 7th Framework Program, and two patient organizations, Cure CMD and the Swiss Foundation for Research on Muscle Diseases (FSRMM), to evaluate the pharmacokinetic profile, safety and tolerability of orally administered omigapil. The clinical Phase I study in pediatric CMD patients will also identify clinical parameters suitable for future efficacy studies.

- **Started development of novel formulation for Raxone (idebenone)**
  Santhera is developing a transmucosal formulation for idebenone as part of its future life cycle management efforts. This novel formulation, for which patent applications have been filed, is aimed at offering improved bioavailability and better convenience for certain patient populations.

Outlook

Santhera's main priorities in the near term are the marketing authorizations of Raxone/Catena for the indications LHON and DMD. In Europe, the regulatory review of the MAA for LHON is ongoing and an opinion from the CHMP is expected in H1 2015. Subject to a positive decision, Raxone will become the first product authorized for the treatment of LHON. Santhera currently prepares to file for regulatory approvals for Raxone/Catena in DMD in the US and EU. Recently, the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for Raxone/Catena for the treatment of DMD.

Santhera expanded its Executive Management and started building a commercial team to prepare the anticipated launch of Raxone in Europe. Marketing efforts will focus on major European markets to be supplemented by national and/or regional distribution agreements. The Company is also evaluating opportunities to commercialize its products in North America.

Santhera believes that, with cash of CHF 14.0 million (March 31, 2015) and the potential to use conditional and authorized capital, it has sufficient financial flexibility to support the development and commercialization of the current pipeline.

Upcoming Events

The Company's Annual Shareholder Meeting will be held on May 11, 2015 in Basel, Switzerland (shareholders will receive a separate invitation).

2014 Full-Year Financial Information

See www.santhera.com/reports for the Santhera 2014 Annual Report.

Condensed consolidated income statement (IFRS, in CHF thousands)	**2014**	2013
Net sales	**2,591**	1,319
Gross profit	**2,392**	**1,179**
Other operating income	**533**	256
Development	**–5,695**	–4,709
Marketing and sales	**–574**	–926
General and administrative	**–4,164**	–3,109
Other operating expenses	**–9**	0
Operating expenses	**–10,442**	–8,744
Operating result	**–7,517**	**–7,309**
Financial result	**–15**	1,549
Income taxes	**–2**	5
Net result	**–7,534**	**–5,755**
Basic and diluted loss per share (in CHF)	**–1.60**	**–1.55**

Condensed consolidated balance sheet (IFRS, in CHF thousands, as of December 31)	**2014**	2013
Cash and cash equivalents	**17,435**	5,044
Noncurrent assets	**4,414**	4,349
Other current assets	**1,096**	343
Total assets	**22,945**	**9,736**
Equity	**17,238**	7,106
Noncurrent liabilities	**2,680**	997
Current liabilities	**3,027**	1,633
Total equity and liabilities	**22,945**	**9,736**

Condensed consolidated cash flow statement (IFRS, in CHF thousands)	**2014**	2013
Operating cash flow	**–6,063**	–6,976
Cash and cash equivalents at January 1	**5,044**	12,283
Cash and cash equivalents at December 31	**17,435**	5,044
Net change in cash and cash equivalents	**12,391**	**−7,239**

Share capital (number of shares with par value of CHF 1, as of December 31)	**2014**	2013
Shares issued	**4,974,492**	3,934,049
Conditional capital for stock options	**659,269**	684,414
Conditional capital for convertible rights	**600,000**	355,000
Authorized capital	**1,500,000**	1,800,000

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 develops Raxone®/Catena® as treatment for patients with Leber Hereditary Optic Neuropathy (LHON), Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS), as well as omigapil for Congenital Muscular Dystrophies (CMD), all areas of high unmet medical need for which no therapies are currently available. For further information, please visit the Company's website www.santhera.com.

Raxone® and Catena® are trademarks of Santhera Pharmaceuticals.

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