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Santhera Reports First Half 2014 Financial Results and Positive Developments in All Strategic Programs

Liestal, Switzerland, September 9, 2014 – Santhera Pharmaceuticals (SIX: SANN) announced today the financial results for the first half year 2014 and reported significant progress for all programs. The Company already generates revenues with Raxone® for the treatment of Leber's Hereditary Optic Neuropathy (LHON) under the French temporary authorization for use (cATU) and named patient programs in certain additional countries. The net cash burn was markedly reduced due to the lean organization, increasing income from product sales and income from share placements.

Commenting on the first six months of 2014, **Thomas Meier, Chief Executive Officer** of Santhera, said, "We made excellent progress on all key programs for Raxone and reached a number of important milestones. In particular, we re-filed the Marketing Authorization Application for Raxone to treat LHON and achieved positive results from our phase III study in Duchenne Muscular Dystrophy."

From a financial standpoint, the recent sale of treasury shares in August, which raised an additional CHF 13.4 million, in conjunction with the increased income from product sales, has substantially improved the cash position and ensures that adequate funds are available to support ongoing development and regulatory projects and to commence marketing preparations for a potential launch of Raxone in Europe.

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 (ANSM) granted a temporary approval (cATU) for Raxone to treat LHON. Santhera is providing Raxone, which is fully reimbursed by a special government program, to an increasing number of LHON patients in France.

- **Collected additional clinical data for Raxone in LHON that substantiates clinical efficacy**
  Visual acuity data collected throughout the first half of 2014 from LHON patients enrolled in an ongoing worldwide Expanded Access Program (EAP) indicate that about 50% of Raxone-treated patients experience a clinically meaningful recovery of vision. Santhera also completed the collaboration with the European Vision Institute Clinical Research Network (EVICR.net) and collected the largest natural history database for this disease. The additional efficacy data from the EAP and the natural history data set were included in the regulatory filing dossier.

- **European Medicines Agency (EMA) validated and started review of Marketing Authorization Application (MAA) for Raxone in LHON**
  Following discussions with several EU member states on the additional clinical efficacy data (from the EAP and natural history data) and the overall content of the revised MAA dossier for LHON, Santhera resubmitted an MAA in May. The filing was validated in early June and the decision by the EMA is expected in 1H 2015. An approval decision would lead to Raxone becoming the first product authorized by the EMA for the treatment of LHON.

- **Reported a successful outcome of the DELOS phase III study in Duchenne Muscular Dystrophy (DMD)**
  In May the first ever positive outcome of a double-blind, placebo-controlled phase III trial in DMD was announced. The study met its primary objective and demonstrated that Raxone/ Catena can delay the loss of respiratory function in patients not taking concomitant glucocorticoid steroids. The preservation of respiratory function is considered of major clinical importance for patients with DMD. The Company plans to present results of the trial at the International Congress of the World Muscle Society in Berlin (DE) in October this year and at the Action Duchenne Conference in London (UK) in November.

- **Initiated a clinical development program with omigapil in Congenital Muscular Dystrophies (CMD) with support from private-public partnership**
  In July the initiation of a phase I program, called CALLISTO, was announced with omigapil in pediatric CMD patients to be conducted by the U.S. National Institutes of Health (NIH). The program received financial support from an EU grant and several patient organizations. Patient enrolment is planned for late this year.

- **Ongoing collaboration with NIH in phase II trial with Catena in Primary Progressive Multiple Sclerosis (PPMS)**
  The company continues to collaborate with the Neuroimmunology Branch of the National Institute of Neurological Disorders and Stroke (NINDS) in a double-blind, placebo-controlled phase II clinical trial (IPPoMS trial) investigating the efficacy of Catena in patients with PPMS.

Financial Highlights:

- **Financing and initial Raxone sales eliminated cash burn during six month period**
  As of June 30, 2014, Santhera had cash and cash equivalents of CHF 5.0 million. Net change in cash and cash equivalents in the first half year of 2014 was reduced to almost zero (TCHF 4; 1H 2013: CHF –4.7 million) as a result of the private share placement with new investor IGLU Group, the sale of shares under the SEDA and increasing income from product sales. Together with the funds received from the recent sale of treasury shares as well as stock option exercises the Company's cash position at the end of August 2014 amounted to CHF 18.9 million.

- **Increasing sales of Raxone**
  In the first six months of 2014, net sales were CHF 0.8 million, mainly driven by the first sales of Raxone for LHON under the cATU in France as well as sales for DMD and LHON patients under Named Patient and Special Access Programs.

- **Reduced operating expenses**
  Operating expenses of CHF 3.9 million (1H 2013: CHF 6.0 million) were comprised of CHF 1.9 million in development, CHF 0.2 million in marketing and sales expense, and CHF 1.8 million in general and administrative expenses. The lower expenses were the result of a continued focus on core program operations. As a result, Santhera narrowed its operating loss to CHF –3.1 million (1H 2013: CHF –4.8 million).

- **Markedly improved net result**
  On a comparable basis, Santhera reported an improved net result of CHF –3.1 million (1H 2013: CHF –3.2 million, which included extraordinary financial income of CHF 1.5 million from the settlement of finance lease liabilities).

Outlook:

Santhera's main priorities in the near term are the successful regulatory filings of Raxone/Catena for LHON and DMD in Europe and the US.

In Europe, the regulatory review for Raxone for **LHON** is underway and a decision is expected in the first half of 2015. Subject to a positive decision from the EMA, Raxone has the potential to become the first product authorized for the treatment of LHON. Based on the data package filed in the European MAA, Santhera will now approach the U.S. Food and Drug Administration (FDA) to discuss a regulatory path to potential U.S. approval of Catena in this indication.

Following the successful outcome of the phase III trial with Raxone/Catena in **DMD**, Santhera also plans to seek regulatory approval for the treatment of DMD in Europe and the U.S. Discussions with European and U.S. regulators have been initiated to identify the most expeditious regulatory approval pathway for this indication.

The company believes that, with the additional CHF 13.4 million, it has sufficient cash to support its currently planned development and regulatory programs and to make necessary preparations, as appropriate, for the commercial introduction of Raxone in Europe.

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 Catena®/Raxone® as treatment for patients with LHON, Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (PPMS) and 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.

Disclaimer / Forward-looking statements

This press release may contain certain forward-looking statements concerning the Company and its business. Such statements involve certain risks, uncertainties and other factors which could cause the actual results, financial condition, performance or achievements of the Company to be materially different from those expressed or implied by such statements. Readers should therefore not place undue reliance on these statements, particularly not in connection with any contract or investment decision. The Company disclaims any obligation to update these forward-looking statements.

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2014 Half Year Financial Information

See www.santhera.com/reports for the Santhera 2014 Interim Report and all unaudited consolidated financial statements.

Condensed interim consolidated income statement (unaudited, IFRS, for half year ended June 30, in CHF thousands)	**1H 2014**	1H 2013
Net sales	**829**	1,127
Gross profit	**754**	1,000
Other operating income	**23**	155
Development	**–1,913**	–3,155
Marketing and sales	**–157**	–754
General and administrative	**–1,790**	–2,048
Operating expenses	**–3,860**	–5,957
Operating result	**–3,083**	–4,802
Financial result	**–10**	1,569
Income taxes	**–1**	7
Net result	**–3,093**	–3,226
Basic and diluted loss per share (in CHF)	**–0.70**	–0.88

Condensed interim consolidated balance sheet (unaudited, IFRS, in CHF thousands)	**June 30, 2014**	Dec. 31, 2013
Cash and cash equivalents	**5,040**	5,044
Noncurrent assets	**4,447**	4,349
Other current assets	**386**	343
Total assets	**9,873**	9,736
Equity	**6,737**	7,106
Noncurrent liabilities	**1,365**	997
Current liabilities	**1,771**	1,633
Total equity and liabilities	**9,873**	9,736

Condensed interim consolidated cash flow statement (unaudited, IFRS, in CHF thousands)	**1H 2014**	1H 2013
Operating cash flow for half year ended June 30	**–2,572**	**–4,119**
Cash and cash equivalents at January 1	**5,044**	12,283
Cash and cash equivalents at June 30	**5,040**	7,572
Net change in cash and cash equivalents	**–4**	–4,711

Share capital (number of shares with par value of CHF 1)	**June 30, 2014**	Dec. 31, 2013
Shares issued	**4,669,000**	3,934,049
Conditional capital for stock options	**764,761**	684,414
Conditional capital for convertible rights	**800,000**	355,000
Authorized capital	**1,500,000**	1,800,000

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