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Santhera Reports Financial Results for the First Half Year and Transitions Towards Product Company

Liestal, Switzerland, September 3, 2015 – Santhera Pharmaceuticals (SIX: SANN) announces the financial results for the first half year 2015 and its transformation into a specialty pharmaceutical company with sustainable revenues from product sales. In June 2015, the Committee for Medicinal Products for Human Use (CHMP) recommended granting a marketing authorization in Europe for Raxone® for the treatment of Leber's Hereditary Optic Neuropathy (LHON) and Santhera expects a final decision from the European Commission (EC) very soon. In parallel, the Company advances Raxone®/Catena® with high priority towards approval in Duchenne Muscular Dystrophy (DMD) in Europe and the USA.

Thomas Meier, PhD, Chief Executive Officer of Santhera, commented on the first six months of 2015: "The recommendation for approval granted in June by the CHMP for Raxone as first treatment for LHON marks the biggest success in our Company's history. Raxone is to become the first treatment approved for any mitochondrial disease and will establish Santhera as leader in mitochondrial medicine. Subject to approval from the EC, we expect to roll out the commercial launch of Raxone in the first countries during the second half of the year, providing the first treatment opportunity to LHON patients who typically remain permanently blind if untreated. Based on the data from the successful Phase III trial in DMD, we continue to prepare for regulatory filings for Raxone/Catena in DMD in the fourth quarter of 2015, both in the United States of America and in Europe."

Christoph Rentsch, Chief Financial Officer of Santhera, added: "We recently raised CHF 27.7 million by the sale of 300,000 registered shares. Combined with the increased income from product sales during the first half this transaction substantially increased the Company's cash position, which at the end of August amounted to CHF 37.0 million. Santhera now has the financial resources to expedite the commercial launch of Raxone for the treatment of LHON in the European Union."

Key Financials in the First Half Year:

- **Preparation for product launch increased cash burn during six-month period**
  As of June 30, 2015, Santhera had cash and cash equivalents of CHF 10.5 million. Net change in cash and cash equivalents in the first half year of 2015 increased to CHF –7.0 million (1H 2014: CHF 0 million) as a result of the ongoing preparation to launch Raxone in Europe.

- **Increase in sales of Raxone**
  In the first six months of 2015, Raxone generated net sales of CHF 1.5 million (1H 2014: CHF 0.8 million), driven by increased product sales for LHON patients under the temporary authorization cATU in France as well as sales under named patient and special access programs.

- **Higher operating expenses due to launch preparations and expanded operations**
  Operating expenses of CHF 7.4 million (1H 2014: CHF 3.9 million) were comprised of CHF 2.8 million in development, CHF 1.4 million in marketing and sales expenses, and CHF 3.1 million in general and administrative expenses (G&A). The increase reflects the hiring of a commercial team for the product launch, higher expenses in development due to preparation of regulatory filings and higher expenses in G&A resulting from the expansion of Santhera's operations. Consequently, the operating loss was CHF –6.0 million (1H 2014: CHF –3.1 million) leading to a net result of CHF –6.2 million (1H 2014: CHF –3.1 million).

Product and Pipeline Highlights:

- **Received positive opinion from the CHMP**
  In June 2015, the CHMP recommended granting a marketing authorization for Raxone for the treatment of visual impairment in adolescent and adult patients with LHON. The formal marketing authorization from the EC for the product is expected shortly.

- **Advanced preparation for product launch**
  In recent months, the Company prepared for a fast and efficient launch of Raxone which is planned for the first countries in coming weeks subject to EC approval.

- **Published outcome of Phase III study in *The Lancet* and prepares for regulatory filings for Raxone/Catena in DMD in Europe and the USA**
  Earlier this year, the positive outcome of the Phase III study was published in *The Lancet* and presented at the annual meeting of the American Academy of Neurology. Based on available data from Santhera's development program with Raxone/Catena in DMD and supplemented by natural history data which we obtain from a collaboration with the Cooperative International Neuromuscular Research Group (CINRG), Santhera plans to submit a New Drug Application (NDA) in the USA and Marketing Authorization Application (MAA) as a variation to the EC approval in LHON. In April 2015, the US FDA has granted Fast Track Designation for Raxone/Catena for the treatment of DMD.

- **Started dosing of first Congenital Muscular Dystrophy (CMD) patient with omigapil and completed patient recruitment in collaborative study with US National Institutes of Health (NIH)**
  The first patient in the CALLISTO Phase I trial was dosed with oral omigapil in a new formulation developed by Santhera for use in children. At the same time, all 20 participants required for the study have been selected, prescreened and randomly assigned to one of the three study cohorts. The study is being conducted at the NIH's National Institute of Neurological Disorders and Stroke in Bethesda (Maryland, USA) and assesses the pharmacokinetics, safety, and tolerability of omigapil in ambulatory and non-ambulatory children affected by either Ullrich or MDC1A subtypes of CMD.

Corporate Highlights:

- **Expanded Executive Management and started building a commercial team**
  In the first half year of 2015, Santhera nominated three senior members of staff to its Executive Management and newly appointed a Chief Commercial Officer and a Chief Financial Officer in a step to prepare the Company for its growth strategy. Simultaneously, the Company started building a commercial team with experience in successfully managing the international commercialization of orphan and niche-market drug products.

Outlook:

In Europe, after approval from the EC, Raxone will be commercially launched in the first countries for the treatment of LHON starting in the second half of 2015.

The Company continues its interactions with regulatory authorities and plans to submit NDA and MAA filings for DMD based on the positive Phase III DELOS trial, the natural history study from the CINRG, and other supportive evidence of efficacy.

The Company believes that, with CHF 37.0 million as of end of August 2015, it has sufficient cash to support the commercial introduction of Raxone in Europe and the currently planned development and regulatory programs.

2015 Half Year Financial Information

See www.santhera.com/reports for Santhera's 2015 Interim Report and all reviewed consolidated financial statements.

Condensed interim consolidated income statement (reviewed, IFRS, for half year ended June 30, in CHF thousands)	**1H 2015**	1H 2014
Net sales	**1,455**	829
Gross profit	**1,296**	754
Other operating income	**23**	23
Development	**–2,846**	–1,913
Marketing and sales	**–1,415**	–157
General and administrative	**–3,101**	–1,790
Operating expenses	**–7,366**	–3,860
Operating result	**–6,047**	–3,083
Financial result	**–162**	–10
Income taxes	**–2**	–1
Net result	**–6,211**	–3,093
Basic and diluted loss per share (in CHF)	**–1.24**	–0.70

Condensed interim consolidated balance sheet (IFRS, in CHF thousands)	**June 30, 2015** **(reviewed)**	Dec. 31, 2014 (audited)
Cash and cash equivalents	**10,476**	17,435
Noncurrent assets	**4,018**	4,414
Other current assets	**4,562**	1,096
Total assets	**19,056**	22,945
Equity	**11,884**	17,238
Noncurrent liabilities	**2,696**	2,680
Current liabilities	**4,476**	3,027
Total equity and liabilities	**19,056**	22,945

Condensed interim consolidated cash flow statement (unaudited, IFRS, in CHF thousands)	**2015**	2014
Operating cash flow for half year ended June 30	**–7,167**	–2,572
Cash and cash equivalents at January 1	**17,435**	5,044
Cash and cash equivalents at June 30	**10,476**	5,040
Net change in cash and cash equivalents	**–6,959**	–4

Share capital (number of shares with par value of CHF 1)	**June 30, 2015** **(reviewed)**	Dec. 31, 2014 (audited)
Shares issued	**5,049,213**	4,974,492
Conditional capital for stock options	**725,279**	604,029
Conditional capital for convertible rights	**950,000**	600,000
Authorized capital	**1,500,000**	1,500,000

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's Hereditary Optic Neuropathy (LHON), Duchenne Muscular Dystrophy (DMD) and primary progressive Multiple Sclerosis (ppMS) and omigapil for Congenital Muscular Dystrophy (CMD), all areas of high unmet medical need. In June 2015, the Committee for Medicinal Products for Human Use (CHMP) recommended granting a marketing authorization in Europe for Raxone® for the treatment of LHON. For further information, please visit the Company's website www.santhera.com.

Raxone® and Catena® are trademarks of Santhera Pharmaceuticals.

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