

## Santhera Reports Strong Top-line Growth for 2018 and the Successful Expansion of its Rare Disease Pipeline

Pratteln, Switzerland, April 29, 2019 – Santhera Pharmaceuticals (SIX: SANN) announces the Group's audited financial results for 2018. The Company reports a 38% increase in net revenues year-on-year to CHF 31.7 million (2017: CHF 22.9 million) from continued growth in sales of its lead product Raxone® for the treatment of Leber's hereditary optic neuropathy (LHON). The operating result of CHF –51.4 million predominantly reflects investments in several ongoing clinical development programs and preparations for regulatory submissions as well as commercial activities. The net result amounted to CHF –54.2 million (2017: CHF –51.5 million), in line with the Company's guidance. Santhera successfully achieved a key objective of broadening its pipeline with clinical stage products for rare diseases with the in-licensing of POL6014 and the option to sub-license *vamorolone*. In the first quarter of 2019, the Company raised new liquid funds of up to CHF 22.1 million and grew sales of Raxone for LHON by 19% year-on-year, reaching CHF 9.2 million (Q1-2018: CHF 7.7 million).

"I am pleased with the progress Santhera has made during the past year. We have achieved excellent sales performance and robust revenue growth with Raxone for the treatment of LHON in 2018 and the continued expansion of our rare disease pipeline with clinical-stage drug candidates," said **Thomas Meier, PhD, CEO of Santhera**. "We have delivered on a strategic objective to broaden our development pipeline by acquiring rights to two highly promising drug candidates in areas with high unmet medical need, proving our ability to in-license high-quality, clinical-stage rare disease assets."

"In addition, we have focused our work on substantially strengthening our data package for *idebenone* in Duchenne muscular dystrophy (DMD) demonstrating clinically relevant patient benefits and sustained, long-term therapeutic efficacy. These new clinical data which collectively supports the potential for *idebenone* to modify the course of respiratory function decline in DMD and thereby delay the time to clinically relevant milestones will be included in our application for Conditional Marketing Authorization (CMA) in Europe. Our strategic priorities for 2019 are the submission of the CMA for Puldysa® (*idebenone*) to treat DMD in Europe, advancing our clinical stage pipeline, in particular *vamorolone* and POL6014, and securing the necessary financial resources to achieve these objectives."

### Strong double-digit sales growth from Raxone® for LHON

In 2018, net revenues from product sales of Raxone for LHON reached CHF 31.7 million which corresponds to a high double-digit growth of 38% year-on-year (2017: CHF 22.9 million). Growth was primarily driven by higher market penetration and additional reimbursement schemes coming into effect. The roll-out of Raxone in the approved indication is progressing as planned with reimbursement and launches in additional countries expected in 2019.

**Operating and net results reflect higher investment in clinical development and for regulatory submissions**

The increase in development expenses to CHF 38.2 million (2017: CHF 26.6 million) reflects the additional efforts for the preparation of the regulatory filing for Puldysa for DMD in Europe, the ongoing late stage clinical studies such as the Phase III SIDEROS trial in DMD, ongoing post-authorization studies for LHON and clinical work with POL6014, which was acquired early in 2018. The Company reports combined marketing and sales expenses of CHF 24.9 million (2017: CHF 28.5 million) and general and administrative expenses of CHF 15.4 million (2017: CHF 14.4 million), generally in line with previous year. In summary, total operating expenses reached CHF 78.7 million (2017: CHF 69.6 million) whereas the operating result remained almost unchanged at CHF –51.4 million (2017: CHF –50.5 million). For the full-year 2018, Santhera reported a net result of CHF –54.2 million (2017: CHF –51.5 million), similar to the previous year's level.

**Cash and cash flows primarily impacted by business development success in expanding the product pipeline**

Santhera expanded its development pipeline in two transactions, acquiring licenses to POL6014 for the treatment of cystic fibrosis and other lung diseases from Polyphor and *vamorolone* with the primary indication DMD from Idorsia, which the Company paid for with a mix of shares and cash. In December 2018, the Company completed an ordinary capital increase with the placement of 3,133,334 new shares and raised gross proceeds of CHF 23.5 million. The proceeds were used in part for the cash payment of the latter transaction, namely to complete the acquisition of the option for the exclusive sub-license to *vamorolone*. As a consequence of these acquisitions, as of December 31, 2018, freely available liquid funds (cash and cash equivalents and short-term financial assets) amounted to CHF 22.0 million (December 31, 2017: CHF 58.2 million). In addition, the Company reported CHF 4.5 million (December 31, 2017: CHF 7.5 million) of restricted cash designated for the interest payments related to the convertible bonds during the first three years.

**Near-term corporate financing initiatives completed**

In April 2019, after the reporting period, Santhera took out a syndicated credit line, which, subject to certain conditions, will provide up to CHF 15.0 million over a time period of nine months. Alongside this, Santhera placed all of the remaining 500,000 registered shares from its existing authorized share capital in a private placement, resulting in aggregate gross proceeds of CHF 7.1 million. Taken together, the proceeds of these near-term financing initiatives provided new liquid funds of up to CHF 22.1 million. In addition, and to fund operations up to the achievement of key milestones expected in 2020, Santhera is considering various options including a capital increase and/or the monetization of certain assets.

**Strong sales growth continues into 2019**

The Company reports sales of Raxone in LHON of CHF 9.2 million in the first three months of 2019. This makes it the strongest quarter so far with a year-on-year sales growth of 19% (Jan-Mar 2018: CHF 7.7 million). The sales increase was driven by higher number of packs sold, new market launches and negotiated reimbursement schemes.

### **Outlook and Guidance**

Based on the performance in the first quarter of 2019 the Company expects continued strong growth and projects net sales of Raxone in the currently approved indication LHON to reach CHF 35-37 million in 2019. The priorities for 2019 are the submission of the Conditional Marketing Authorization (CMA) application for Puldysa to treat DMD in Europe, advancing the other clinical stage candidates in our pipeline, particularly *vamorolone* and POL6014, and securing the necessary financial resources to achieve these objectives.

### **Annual Report**

The Santhera Annual Report 2018 is available for download on the Company's website at [www.santhera.com/investors-and-media/investor-toolbox/financial-reports](http://www.santhera.com/investors-and-media/investor-toolbox/financial-reports).

### **Upcoming Events**

The Annual General Meeting of Santhera will be held on May 28, 2019, in Basel, Switzerland (shareholders will receive a separate invitation). The invitation can be downloaded from [www.santhera.com/investors-and-media/investor-toolbox/shareholder-meetings](http://www.santhera.com/investors-and-media/investor-toolbox/shareholder-meetings).

<b>2018 Full-year Financial Information</b>
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Santhera's 2018 Annual Report see [www.santhera.com/investors-and-media/investor-toolbox/financial-reports](http://www.santhera.com/investors-and-media/investor-toolbox/financial-reports).

<b>Condensed consolidated income statement</b> (IFRS, in CHF thousands)	<b>2018</b>	2017
Net sales	<b>31,657</b>	22,943
Cost of goods sold (of which amortization intangible assets: 2018 -3,039 / 2017 -3,039)	<b>-4,702</b>	-4,104
Development	<b>-38,240</b>	-26,561
Marketing and sales	<b>-24,884</b>	-28,522
General and administrative	<b>-15,365</b>	-14,416
<b>Operating expenses</b>	<b>-78,687</b>	-69,563
<b>Operating result</b>	<b>-51,420</b>	-50,454
Financial result	<b>-2,444</b>	-821
Income taxes	<b>-322</b>	-257
<b>Net result</b>	<b>-54,186</b>	-51,532
Basic and diluted loss per share (in CHF)	<b>-7.86</b>	-8.22

<b>Condensed consolidated balance sheet</b> (IFRS, in CHF thousands)	<b>2018</b>	2017
Cash and cash equivalents	<b>21,971</b>	45,195
Financial assets short-term	<b>0</b>	13,011
Other current assets	<b>21,112</b>	19,402
Noncurrent assets	<b>67,211</b>	32,172
<b>Total assets</b>	<b>110,294</b>	109,780
Equity	<b>27,829</b>	32,256
Noncurrent liabilities	<b>62,756</b>	64,278
Current liabilities	<b>19,709</b>	13,246
<b>Total equity and liabilities</b>	<b>110,294</b>	109,780

<b>Condensed consolidated cash flow statement</b> (IFRS, in CHF thousands)	<b>2018</b>	2017
Operating cash flow	<b>-37,865</b>	-39,633
Investing cash flow	<b>-5,909</b>	-22,239
Financing cash flow	<b>20,644</b>	57,108
Cash and cash equivalents at January 1	<b>45,195</b>	49,815
Cash and cash equivalents at December 31	<b>21,971</b>	45,195
<b>Net change in cash and cash equivalents</b>	<b>-23,224</b>	-4,620

<b>Share capital</b> (number of shares with par value of CHF 1)	<b>2018</b>	2017
Shares issued	<b>10,664,563</b>	6,288,555
Conditional capital for equity rights	<b>687,552</b>	691,302
Conditional capital for convertible rights	<b>930,000</b>	930,000
Authorized capital	<b>500,000</b>	1,500,000

**About Santhera**

Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare and other diseases with high unmet medical needs. The portfolio comprises clinical stage and marketed treatments for neuro-ophthalmologic, neuromuscular and pulmonary diseases. Santhera's Raxone® (idebenone) is authorized in the European Union, Norway, Iceland, Liechtenstein, Israel and Serbia for the treatment of Leber's hereditary optic neuropathy (LHON) and is currently commercialized in more than 20 countries. For further information, please visit [www.santhera.com](http://www.santhera.com).

*Raxone® and Puldysa® are trademarks of Santhera Pharmaceuticals.*

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