

Santhera Announces Financial Results for the First Nine Months of 2018

Pratteln, Switzerland, December 12, 2018 – Santhera Pharmaceuticals (SIX: SANN) reports financial results for the nine months ended September 30, 2018, and confirms its guidance and positive outlook.

Santhera is on track to meet its strategic and financial goals for 2018. The Company further delivered on its active in-licensing strategy for high quality, late-stage rare disease assets by acquiring an exclusive sub-license option to vamorolone, the first-in-class dissociative steroid currently in development for the treatment of Duchenne muscular dystrophy (DMD). On the commercial side, the Company has further grown sales of the revenue-generating product Raxone® for the treatment of Leber's hereditary optic neuropathy (LHON) and expects to meet its 2018 full-year sales guidance in the range of CHF 30-32 million.

Santhera continued its business expansion and grew sales to CHF 23.6 million in the first nine months of 2018 which corresponds to a 45% growth rate (compared to the same period in the previous year). Development expenses increased by 49% compared to the same prior-year period, primarily driven by clinical development activities for POL6014 for the treatment of cystic fibrosis (CF) and regulatory work in preparation of filings for idebenone in the indication DMD. Efficient use of resources resulted in lower marketing and sales expenses (–6%) and a slower rise in general and administrative expenses (+18%). Taken together, this contributed to an increase of operating expenses by 20% compared to the same period of last year. In summary, Santhera closed the nine-month period under review with a net result of CHF –39.9 million (Jan.-Sept. 2017: CHF –33.3 million).

Financial highlights:

- Sales of CHF 23.6 million (Jan.-Sept. 2018), reflecting an increase by 45% compared to same period last year (Jan.-Sept. 2017: CHF 16.3 million)
- Operating expenses of CHF 57.2 million (Jan.-Sept. 2017: CHF 47.7 million)
- Operating result of CHF –37.2 million (Jan.-Sept. 2017: CHF –34.2 million), leading to a net result of CHF –39.9 million (Jan.-Sept. 2017: CHF –33.3 million)
- Cash, cash equivalents and short-term financial assets of CHF 25.4 million (as of September 30, 2018)
- 2018 full-year sales guidance of CHF 30-32 million confirmed

Corporate highlights:

- Agreement with Idorsia Ltd under which Santhera is to acquire the option to an exclusive sub-license of the first-in-class dissociative steroid vamorolone in all indications and all countries worldwide except Japan and South Korea (November 20, 2018)
- Idorsia became largest shareholder in Santhera
- Extraordinary General Meeting (EGM) approval of the capital increase in connection with the vamorolone sub-license agreement (December 11, 2018)

Operational highlights (July 2018 to present day):

- Analysis of new data linking study findings with idebenone in DMD to clinically relevant patient benefits for inclusion in regulatory submissions in Europe and the U.S.
- Positive opinion on orphan drug designation received from European regulators for POL6014 for the treatment of CF
- Start of a Phase Ib/IIa multiple ascending dose (MAD) trial with POL6014 in patients with CF

Revenue Guidance:

Santhera will continue to grow its international business, advance its pipeline programs and actively proceed with business development initiatives to expand its late stage product portfolio. Based on its sales performance to date this year, the Company confirms its previous guidance and expects to reach a full-year sales for Raxone® in the range of CHF 30-32 million in 2018.

Note

The financial statements as of and for the nine months ended September 30, 2018 have been prepared and are being published exceptionally on the occasion of and in connection with the capital increase proposed to and approved by the EGM held on December 11, 2018. Santhera publishes results in line with the disclosure requirements of the SIX Swiss Exchange. Santhera does not plan on publishing quarterly reports in the future.

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 and Israel 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.

Raxone® is a trademark of Santhera Pharmaceuticals.

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| Financial Information for the nine months ended September 30, 2018 |
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Santhera's Interim Condensed Report January to September 2018 can be viewed at www.santhera.com/investors-and-media/investor-toolbox/financial-reports.

| Condensed interim consolidated income statement (unaudited, IFRS, for nine months ended September 30, in CHF thousands) | Jan.-Sept. 2018 | Jan.-Sept. 2017 |
|---|------------------------|-----------------|
| Net sales | 23,634 | 16,347 |
| Cost of goods sold (of which amortization intangible assets: 2018 -2,279 / 2017 -2,279) | -3,606 | -3,028 |
| Development | -27,098 | -18,168 |
| Marketing and sales | -18,637 | -19,909 |
| General and administrative | -11,292 | -9,573 |
| Operating expenses | -57,196 | -47,718 |
| Operating result | -37,167 | -34,156 |
| Financial result | -2,376 | -72 |
| Income taxes | -380 | 895 |
| Net result | -39,923 | -33,333 |
| Basic and diluted loss per share (in CHF) | -6.19 | -5.32 |

| Condensed interim consolidated balance sheet (IFRS, in CHF thousands) | Sept. 30, 2018 (unaudited) | Dec. 31, 2017 (audited) |
|---|--------------------------------------|----------------------------|
| Cash and cash equivalents | 19,654 | 45,195 |
| Financial assets short-term | 5,735 | 13,011 |
| Other current assets | 20,076 | 19,402 |
| Noncurrent assets | 33,307 | 32,172 |
| Total assets | 78,772 | 109,780 |
| Equity | 4,803 | 32,256 |
| Noncurrent liabilities | 62,141 | 64,278 |
| Current liabilities | 11,828 | 13,246 |
| Total equity and liabilities | 78,772 | 109,780 |

| Condensed interim consolidated cash flow statement (unaudited, IFRS, in CHF thousands) | 2018 | 2017 |
|--|----------------|---------|
| Operating cash flow for nine months ended September 30 | -32,959 | -31,883 |
| Investing cash flow for nine months ended September 30 | 8,653 | -21,384 |
| Financing cash flow for nine months ended September 30 | -1,155 | 57,101 |
| Cash and cash equivalents at January 1 | 45,195 | 49,815 |
| Cash and cash equivalents at September 30 | 19,654 | 53,743 |
| Net change in cash and cash equivalents | -25,541 | 3,928 |

| Share capital (number of shares with par value of CHF 1) | Sept. 30, 2018 (unaudited) | Dec. 31, 2017 (audited) |
|--|--------------------------------------|----------------------------|
| Shares issued | 6,527,479 | 6,288,555 |
| Conditional capital for stock options | 691,302 | 691,302 |
| Conditional capital for convertible rights | 930,000 | 930,000 |
| Authorized capital | 1,500,000 | 1,500,000 |

Disclaimer / Forward-looking statements

This communication does not constitute an offer or invitation to subscribe for or purchase any securities of Santhera Pharmaceuticals Holding AG. This publication 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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