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Ad hoc announcement pursuant to Art. 53 LR

*A conference call will be held today at 14:30 CEST, 13:30 BST, 08:30 EDT. Details are at the end of this statement.*

# Santhera Reports 2021 Annual Results

- **Revenue from contracts with customers of CHF -1.6 million (2020: CHF 15.0 million)**
- **Operating result of CHF -56.9 million (2020: CHF -53.1 million) and net result of CHF -55.5 million (2020: CHF -67.7 million)**
- **Cash and cash equivalents of CHF 21.2 million as of December 31, 2021**
- **Recent financing is expected to provide funding through Q1-2023 or up to the earliest expected approval time for vamorolone in the U.S.**
- **Key regulatory milestones reached with vamorolone; rolling submission to the U.S. FDA to be completed by end of June 2022**

**Pratteln, Switzerland, June 10, 2022 – Santhera Pharmaceuticals (SIX: SANN) announces the Company's audited financial results for 2021 and reports on progress in securing financing and advancing its lead compound vamorolone for the treatment of Duchenne muscular dystrophy (DMD) towards U.S. regulatory approval.**

"Our overarching goal for 2021 was to advance our lead therapy candidate vamorolone for Duchenne muscular dystrophy (DMD) towards registration and approval—and we are very pleased to have delivered on key milestones as promised. Looking forward, we are preparing for the next phase of Santhera's development as we get the Company ready for the potential market entry of vamorolone, initially in the U.S.," said **Dario Eklund, CEO of Santhera**. "Although vamorolone is our main strategic focus, we also saw progress with lonodelestat, our second clinical development candidate targeting pulmonary indications, and, after successful completion of a Phase 1b trial, Phase 2a trials in cystic fibrosis and an acute pulmonary indication are to start in H2-2022. Financing continues to remain a priority. Very recently, we closed financing agreements which are expected to extend the Company's cash reach into Q1-2023 or up to the approval of vamorolone in the U.S., anticipated in Q1-2023 subject to priority review being granted."

## FINANCIAL PERFORMANCE & FINANCING OUTLOOK

- Net revenue from contracts with customers of CHF -1.6 million
- Operating expenses of CHF 51.9 million, reduced by 11%
- Net result of CHF -55.5 million (2020: CHF -67.7 million)
- Cash and cash equivalents of CHF 21.2 million (December 31, 2021)
- Operating cash flow CHF -37.4 million

**2021 full-year net revenues**

In 2021, Santhera reported net revenue of CHF -1.6 million (2020: CHF 15.0 million). Revenue from out-licensing transactions and net sales to licensing partners remained fairly stable year-on-year. The negative net sales in the EU in 2021 are attributable to a CHF 10.8 million adjustment to net sales of Raxone® due to uncertainties and status of ongoing negotiations around pricing reimbursement in France.

Santhera continues to supply Raxone in France following the outlicensing and transfer to Chiesi Group in 2019 outside of France and North America. Based on the agreement with the French authorities, Santhera has supplied Raxone free of charge from August 2021 following its removal from the list of reimbursed drugs. Reimbursement discussions are ongoing and these are expected to be supported by the recently successfully completed Phase 4 trials LEROS and PAROS with Raxone in the rare ophthalmic indication Leber's hereditary optic neuropathy (LHON). These results also support further reimbursement negotiations in Europe and increase product visibility which could lead to partnering interests for North America.

**Cost of goods sold**

Cost of goods sold amounted to CHF 3.8 million (2020: CHF 10.4 million). The reduction reflects the inclusion of an inventory impairment of CHF 6.0 million in 2020 related to the discontinuation of Puldysa. Ongoing cost of goods represents continuing supply of Raxone.

**Operating expenses**

Operating expenses of CHF 51.9 million (2020: CHF 58.4 million) were 11% lower due to reduced expenses for development, marketing and sales, and general administrative purposes following the termination of the Puldysa program in 2020 and the subsequent restructuring.

Development expenses were CHF 29.7 million and CHF 34.2 million for the year ended December 31, 2021, and 2020, respectively. The decrease in expenses was primarily due to lower contract research organization expenses and other third-party clinical trial expenses following the termination of the Puldysa Phase 3 SIDEROS study, offset by increased expenses to support the development of vamorolone to completion of the VISION-DMD study, in addition to a reduction in staff costs following organizational restructuring.

Marketing and sales expenses were CHF 9.3 million and CHF 11.5 million for the year ended December 31, 2021 and 2020, respectively. The decrease was primarily a result of the ceasing of Puldysa activities following the termination of the program announced in October 2020. Ongoing expenses relate to pre-commercialization activities for vamorolone and meeting ongoing obligations in relation to Raxone outlicensed to Chiesi Group.

General and administrative expenses remained largely unchanged with CHF 12.7 million and CHF 12.4 million for the year ended December 31, 2021 and 2020, respectively.

**Financial income and expenses**

The net financial income of CHF 2.1 million (2020: expense of CHF 14.4 million) reflects effects of the exchange of the 17/22 convertible bond which were partially offset by the costs associated for financing.

**Net result**

The net result for the year ended December 31, 2021, was a loss of CHF 55.5 million, or CHF -1.62 per share, compared to a net loss of CHF 67.7 million or CHF -5.08 per share for the year ended December 31, 2020.

**Cash flow and cash balance**

As of December 31, 2021, the Company had cash and cash equivalents of CHF 21.2 million compared to CHF 12.4 million as of December 31, 2020.

Net cash used in operating activities was CHF 37.4 million for the twelve months ended December 31, 2021, compared to CHF 43.5 million for the twelve months ended December 31, 2020.

**Shareholders' equity**

Total consolidated equity as of December 31, 2021, amounted to CHF 1.3 million compared to a net equity deficit of CHF 6.4 million as of December 31, 2020.

**Recent financing activities and outlook**

In order to provide Santhera with additional fundraising flexibility, the Company has issued and plans to issue additional treasury shares. During March, 2022, post period end, Santhera issued 18,600,000 additional treasury shares with a nominal value of CHF 1 each consisting 3,100,000 shares from authorized capital and 15,500,000 shares as an ordinary capital increase. As a result, Santhera's issued share capital currently amounts to CHF 73,725,702. This number includes 4,328 shares that have been issued from conditional capital which have not yet been updated in the commercial register. Santhera expects to hold 21,768,585 shares as treasury shares until market conditions permit for a favorable financing transaction. In addition, with the exception of the aforementioned 4,328 shares, Santhera updated its articles of association to reflect past share issuances out of the conditional capital for employee participation and out of the conditional capital for financings.

Concurrently with the ordinary capital increase and as decided by the EGM on December 15, 2021, Santhera's authorized capital has increased from CHF 24,203,905 to CHF 34,203,905 and its conditional capital for financing has increased from CHF 21,374,664 to CHF 31,374,664.

On June 2, 2022, the Company entered into an amendment to the timing of an upcoming milestone payment to partner ReveraGen, thereby reducing near-term financial obligations of the Company by CHF 20 million. The Company also upsized its existing financing arrangement with certain funds managed by Highbridge Capital Management, LLC ("Highbridge") which will provide up to CHF 40 million of additional financing. Santhera expects the combination of these events to extend its liquidity runway into 2023 or up to approval of vamorolone in the U.S. which, subject to priority review being granted, is expected in Q1-2023. The first tranche of CHF 20 million was drawn on June 3, 2022.

**Funding outlook**

Santhera has a significant amount of treasury shares from past EGM authorization which are available for future placement which may include existing shareholders' subscription rights, depending on market conditions. This, in combination with cash balances as of December 31, 2021, of CHF 21.2 million and recently increased facilities, provides flexibility to secure sufficient funding beyond FDA approval of vamorolone in DMD. In order to reach profitability with vamorolone in DMD, which is currently expected,

at the earliest, during H2-2024, Santhera estimates that the Company will need to secure an additional CHF 40-50 million to fund its operations, including U.S. approval milestone payments and its debt service. Following the recent financing, this is around 50% less than the previously communicated funding need of about CHF 100 million.

In parallel, Santhera is evaluating a number of different options to secure additional financing for the Company which besides equity-based funding also includes a rights offering, debt financing, royalty financing, standby equity distribution agreement as well as the monetization of assets.

## PIPELINE MILESTONES AND PROGRESS REVIEW

Throughout 2021 and into 2022, Santhera made progress on all fronts. Santhera realigned its organization, initiated the NDA submission with vamorolone in DMD to the U.S. FDA, further advanced its core pipeline projects and started expanding its U.S. operations in view of a near-term launch.

**Lead pipeline candidate vamorolone on track for expected first market entry in early 2023**
Vamorolone is under joint development by ReveraGen and Santhera for Duchenne muscular dystrophy (DMD) patients who require anti-inflammatory and muscle preserving treatment comparable to current standards of care but with an improved safety and tolerability profile to overcome side effects that often lead to premature treatment discontinuation in real world practice.

Furthermore, recent analyses comparing the long-term efficacy and safety of vamorolone to deflazacort and prednisone, the current standard of care, indicate that vamorolone has comparable efficacy but has a unique bone sparing profile that resulted in no stunting of growth as well as fewer and less severe spinal fractures. This data will shortly be presented at several medical and scientific congresses.

Following a successful pre-NDA meeting with the FDA, Santhera initiated a rolling NDA submission in the U.S. in March 2022 which the Company expects to complete by the end of June 2022. Commercial launch would start in the U.S., subject to approval which is expected in Q1-2023 at the earliest, followed by Europe where a marketing authorization application (MAA) submission is planned for Q3-2022.

**Establishing market readiness and transitioning Santhera to the commercial stage**
Following the restructuring in 2020, Santhera used 2021 to embark on the path forward with a focus on preparations for the transition to a commercial stage company. Over the coming months, the Company plans to expand its workforce in connection with the preparations for a potential launch of vamorolone in DMD in the U.S. and later in the largest European countries and to support our planned development of vamorolone in other indications and clinical development of lonodelestat.

**Focused pipeline approach to maximize the potential of our development candidates**
Although vamorolone is the main strategic focus, the Company also saw progress in the other pipeline assets. In 2021, Santhera continued the development of lonodelestat, its second clinical development candidate targeting pulmonary indications. In March 2021, Santhera announced promising clinical trial data from a Phase 1b trial of lonodelestat in cystic fibrosis (CF). Next stage Phase 2a trials in CF and an acute pulmonary indication are to start in H2-2022.

Post-period end, Santhera has signed strategic agreements in the rare disease space further exploiting the potential of its pipeline products. In January 2022, the Company entered into an exclusive license agreement with Sperogenix Therapeutics, a China-based company specializing in orphan diseases, for the Greater China area. Under this agreement, Sperogenix has in-licensed vamorolone for rare disease indications for a total consideration of up to USD 124 million and plans to initiate a regulatory filing for vamorolone for DMD in China upon US FDA approval which could lead to market entry in China as early as in 2024. In February 2022, Santhera signed a gene therapy agreement with SEAL Therapeutics which will further develop a gene therapy approach intended for the treatment of congenital muscular dystrophy in exchange for payments based on future proceeds of SEAL Therapeutics.

Additional details on the pipeline products and operational progress can be found in the Annual Report 2021.

**Annual Report**
The Santhera Annual Report 2021 is available for download on the Company's website at www.santhera.com/financial-reports.

**Annual General Meeting 2022**
Pursuant to Art. 27 para. 1 of the COVID-19 Ordinance 3, companies may provide that their shareholders exercise their rights solely by way of giving voting instructions to the independent proxy (unabhängiger Stimmrechtsvertreter). On this basis, the Company hereby mandates that all shareholders exercise their rights at the ordinary General Meeting of shareholders solely via the independent proxy. There is no possibility to attend the AGM in person.

The invitation which will be sent to registered shareholders can be downloaded from www.santhera.com/share-bondholder-meetings. Information on how to issue power of attorney and instructions to the independent proxy, electronically or in writing, can be found on page 11 of this invitation.

**Conference Call**
Santhera will host a conference call on June 10, 2022, at 14:30 CEST / 13:30 BST / 08:30 EDT. CEO Dario Eklund, CFO Andrew Smith and CMO Dr. Shabir Hasham will discuss the 2021 financial results and comment on ongoing corporate developments. Participants are invited to call one of the following numbers 10-15 minutes before the conference call starts (no dial-in code is required):

| | |
|---|---|
| Switzerland/Europe: | +41 58 310 50 00 |
| United Kingdom: | +44 207 107 06 13 |
| USA: | +1 631 570 56 13 |

A replay will be accessible at https://www.santhera.com/ad-hoc-news from about two hours after the call has ended.

## 2021 Full-year Financial Information

Santhera's 2021 Annual Report can be accessed at www.santhera.com/financial-reports.

| **Condensed consolidated income statement** (IFRS, in CHF thousands) | **2021** | 2020 |
|---|---|---|
| Net sales | **-4,963** | 11,252 |
| Revenue from out-licensing transactions | **1,126** | 1,597 |
| Net sales to licensing partner | **2,242** | 2,159 |
| **Revenue from contracts with customers** | **-1,595** | 15,008 |
| Cost of goods sold (of which amortization intangible assets: 2021 -3,040 / 2020 -3,039) | **-3,767** | -10,431 |
| Other operating income | **346** | 694 |
| Development | **-29,715** | -34,228 |
| Marketing and sales | **-9,332** | -11,474 |
| General and administrative | **-12,725** | -12,440 |
| **Operating expenses** | **-51,872** | -58,347 |
| **Operating result** | **-56,888** | -53,076 |
| Financial result | **2,171** | -14,380 |
| Income taxes | **-809** | -203 |
| **Net result** | **-55,526** | -67,659 |
| Basic and diluted net result per share (in CHF) | **-1.62** | -5.08 |

| **Condensed consolidated balance sheet** (IFRS, in CHF thousands) | **2021** | 2020 |
|---|---|---|
| Cash and cash equivalents | **21,208** | 12,411 |
| Other current assets | **3,433** | 5,312 |
| Noncurrent assets | **66,476** | 70,964 |
| **Total assets** | **91,117** | 88,687 |
| Equity | **1,328** | -6,354 |
| Noncurrent liabilities | **57,007** | 65,972 |
| Current liabilities | **32,782** | 29,069 |
| **Total equity and liabilities** | **91,117** | 88,687 |

| **Condensed consolidated cash flow statement** (IFRS, in CHF thousands) | **2021** | 2020 |
|---|---|---|
| Cash flow from/(used in) operating activities | **-37,359** | -43,510 |
| Cash flow from/(used in) investing activities | **69** | 1,563 |
| Cash flow from/(used in) financing activities | **46,022** | 23,150 |
| Cash and cash equivalents at January 1 | **12,411** | 31,358 |
| Cash and cash equivalents at December 31 | **21,208** | 12,411 |
| **Net increase/(decrease) in cash and cash equivalents** | **8,797** | -18,947 |

| **Share capital** (number of shares with par value of CHF 1) | **2021** | 2020 |
|---|---|---|
| Shares issued | **54,607,810** | 19,429,696 |
| Treasury shares | **5,019,879** | 1,580,063 |
| Conditional capital for equity rights | **5,425,677** | 687,052 |
| Conditional capital for convertible rights | **21,878,228** | 1,104,658 |
| Authorized capital | **27,303,905** | 2,080,709 |

**Corporate calendar**
June 30, 2022 Annual General Meeting

**About Santhera**
Santhera Pharmaceuticals (SIX: SANN) is a Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare neuromuscular and pulmonary diseases with high unmet medical need. Santhera has an exclusive license for all indications worldwide to vamorolone, a dissociative steroid with novel mode of action, which was investigated in a pivotal study in patients with DMD as an alternative to standard corticosteroids. The Company plans to complete the rolling submission of its filing for approval for vamorolone with the U.S. FDA in June 2022. The clinical stage pipeline also includes lonodelestat to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases. Santhera out-licensed rights to its first approved product, Raxone® (idebenone), outside North America and France for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. For further information, please visit www.santhera.com.

*Raxone® is a trademark of Santhera Pharmaceuticals.*

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