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Ad hoc announcement pursuant to Art. 53 LR

A conference call will be held on September 12, 2024, at 14:30 CEST / 13:30 BST / 08:30 EDT. Details are at the end of this news release.

Santhera Announces Half-Year 2024 Financial Results and Provides Corporate Update

- Revenue from contracts with customers of CHF 14.1 million (H1-2023: CHF 3.9 million)
- Operating result of CHF -17.7 million (H1-2023: CHF -20.3 million) and net result of CHF -15.3 million (H1-2023: CHF -23.3 million)
- AGAMREE® (vamorolone) launched in Germany and Austria as first European markets; North America partner has launched in the U.S.
- Approval of AGAMREE in the UK for the treatment of Duchenne muscular dystrophy (DMD); new drug application (NDA) in DMD under regulatory priority review in China
- Cash and cash equivalents of CHF 16.5 million (June 30, 2024); bolstered by financing of up to CHF 69 million (closed in August 2024) to provide funding into 2026 when cash flow break-even is expected
- Business now fully focused on European commercialization and further geographic expansion of AGAMREE in DMD

Pratteln, Switzerland, September 12, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces the Company's financial results for the six months ended June 30, 2024, reports on progress with AGAMREE® (vamorolone) for the treatment of Duchenne muscular dystrophy (DMD) and provides updates on its corporate and financing initiatives.

"We are extremely encouraged by the strong early uptake of AGAMREE in Germany and Austria, which has exceeded our expectations. Our North American partner, Catalyst Pharmaceuticals, has also seen a successful launch in the U.S., further validating the potential of AGAMREE in treating DMD," said **Dario Eklund, CEO of Santhera**. "While we are delaying the decision on a new indication for AGAMREE until at least the end of 2025 to allow Catalyst to conduct additional exploratory clinical work, this strategic pause gives us an exciting opportunity. We are reallocating resources and are intensifying our efforts in DMD by expanding our launch into the Nordics, Portugal, and Ireland, hereby retaining full control of all the markets in western Europe. Additionally, we have decided to invest in additional studies to further strengthen the evidence behind AGAMREE's differentiated safety profile and drive continued growth in the DMD space."

BUSINESS AND CORPORATE UPDATE

Half-year 2024 key events and post-period events

- AGAMREE approved in the UK for the treatment of DMD, following prior approvals in the U.S. and EU
- The UK MHRA, in accordance with EU regulators, acknowledged safety benefits of AGAMREE with regards to preserving bone health and maintaining growth compared to standard of care corticosteroids
- Launches of AGAMREE in Germany and Austria (by Santhera) and the U.S. (by Catalyst Pharmaceuticals) as first markets, experiencing strong market uptake
- Preparations for market entry are advancing across Europe, with pricing negotiations currently underway
- Expansion of self-commercialization reach (to include the Nordics, Ireland and Portugal) and new distribution agreements established, representing coverage of all of EU and some non-EU markets in Europe
- Priority review for AGAMREE NDA in DMD granted by China's regulatory authority; early access program started by Sperogenix

AGAMREE approved across the U.S., EU and UK—NDA under priority review in China

On January 11, 2024, the Medicines and Healthcare products Regulatory Agency (MHRA) in the UK approved AGAMREE for the treatment of DMD, following earlier approvals in the U.S. (by FDA on October 26, 2023) and the European Union (by the European Commission on December 18, 2023). AGAMREE became the first DMD treatment approved across these three territories. In the EU, AGAMREE is the first and only approved medication for treating all patients from age 4 years with DMD.

The European Medicines Agency (EMA) and the MHRA acknowledged clinically important safety benefits of AGAMREE with regards to maintaining normal bone metabolism, density and growth compared to standard of care corticosteroids, while demonstrating similar efficacy.

In March 2024, the National Medical Products Administration (NMPA) in China accepted the new drug application (NDA) filing for AGAMREE in DMD for patients aged 4 years and older, incorporating it into both the Priority Review Program and the Breakthrough Therapy Program. Subject to a positive review outcome, approval could be obtained by Q1-2025.

First launches in Germany and the U.S. met with strong market acceptance

The first market launch of AGAMREE (available as a 40 mg/ml oral suspension) for the treatment of DMD occurred on January 15, 2024, in Germany, where around 2,300 patients are affected by DMD. In the following month, AGAMREE became available in Austria. The reception in both markets has been very positive, evidenced by strong demand and proactive inquiries from patients and caretakers. Currently, after only a few months of availability, AGAMREE has been prescribed to around 300 patients in Germany and Austria.

In March 2024, Catalyst Pharmaceuticals, Inc. (NASDAQ: CPRX), executed the U.S. commercial launch of AGAMREE and reported yielding solid results and surpassing initial expectations by the end of June 2024. Catalyst Pharmaceuticals holds an exclusive license for AGAMREE for North America. Further, in July 2024, Catalyst entered into an exclusive license, supply, and commercialization agreement for AGAMREE with a partner in Canada, marking a pivotal strategic milestone in expanding the product's North American footprint.

Paid early access programs started in first countries—broader interest expressed

Santhera received a number of named patient requests from European as well as global markets and is exploring sustainable supply mechanisms with a selection of partners. Meanwhile, programs have started in Spain and China.

In June, Santhera started a paid-for named patient program (NPP) in Spain. Under a well-defined regulatory framework, with the approval of the national as well as regional regulatory bodies, this allows patients with serious or life-threatening conditions to access medicines, even before they are commercially available in the country.

In China, partner Sperogenix Therapeutics has launched a paid-for early access program (EAP) for AGAMREE for patients with DMD. In April 2024, the Hainan Medical Products Administration (HMPA) authorized the EAP for AGAMREE in China, where approved treatments are currently unavailable, based on local policies, AGAMREE's existing overseas approvals (U.S., EU, UK) and its demonstrated ability to address urgent clinical needs in DMD. The EAP started in the Bo'ao Lecheng Pilot Zone, located in Hainan Province, in mid-May, where strong interest has been received and approximately 70 patients are currently on active treatment with AGAMREE.

Pre-commercialization measures advancing across Europe—pricing negotiations ongoing

Santhera plans to make AGAMREE available to patients across Europe. After Germany and Austria, the build-up of a core commercial organization is well advanced in UK, France, Italy, Spain, and Benelux. Activities surrounding market access, stakeholder and key opinion leader engagement in the target countries advanced throughout the period under review. Across key European markets, Santhera is currently engaged in various national processes of health technology assessments (HTA), pricing negotiations, and reimbursement decisions. In Germany, considering that Santhera launched AGAMREE on January 15, 2024, the pricing negotiations are expected to be concluded by January 15, 2025. Owing to the complex and lengthy nature of these evaluations, anticipated timelines for upcoming market entries are likely to be slightly later than previously indicated. The UK is expected to be concluded later this year, France and Spain by mid-2025 and Italy by late 2025. Meanwhile, mid-sized European countries will progress in parallel with the big five markets.

Self-marketing territory expanded—additional distribution agreements secured

Market uptake in the first launch countries has been strong and exceeded Santhera's expectations. On this basis, Santhera has decided to expand its self-marketing strategy for AGAMREE to include the Nordic countries (Denmark, Sweden, Finland, Norway, and Iceland), along with Portugal and Ireland, and the organizational build-up has started. This approach reflects confident economic projections, while ensuring that margins are kept in-house, and allows the Company to maximize value and revenue potential from Agamree in DMD.

For all other markets in Europe, Santhera has signed up **GENESIS Pharma** as its distribution partner. GENESIS Pharma, with domiciles in Greece and Cyprus, is a regional biopharma company specialized in the commercialization of innovative medicines targeting severe and rare diseases in Central and Eastern Europe. The company will market AGAMREE in DMD in Greece, Cyprus, Malta, Romania, Bulgaria, Slovenia, Croatia, Poland, Czech Republic, Hungary, Slovakia, Lithuania, Latvia, Estonia, Serbia, North Macedonia, Bosnia & Herzegovina, Montenegro, Albania, and Kosovo.

A supply and distribution agreement has also been signed with **Megapharm Ltd**. Megapharm is a leading specialty marketing and distribution company in Israel's healthcare sector and will market AGAMREE for the treatment of DMD in Israel and the Palestinian territories.

In addition, Santhera has entered into discussions with multiple potential partners for additional territories outside of Europe.

Clinical programs with AGAMREE

AGAMREE has been developed to provide an anti-inflammatory and muscle preserving treatment with a favorable safety and tolerability profile as an alternative to the current standard of care with glucocorticoids. In addition to long-term efficacy and safety data with AGAMREE, recent publications and presentations further characterized AGAMREE's differentiated profile mainly with regard to bone health.

Clinical studies with AGAMREE in *Duchenne muscular dystrophy (DMD)* were initiated to investigate its effects in a broader patient age group and additional clinical work was started to differentiate the safety profile over the next couple of years. The clinical development program for AGAMREE until now included patients 4 to <7 years old and, as part of the pediatric investigational plan (PIP) requested by EMA, a new Phase 2 study aims at collecting information on AGAMREE outside this age range through inclusion of patients starting at an age of 2 years and up to 18 years. In addition, a Phase 1 study in healthy volunteers aims to confirm AGAMREE's mineralocorticoid antagonistic properties, a key mechanism in current standard therapies to slow cardiomyopathy progression, further distinguishing AGAMREE as the only corticosteroid that may offer early cardioprotective benefits alongside its proven efficacy in DMD.

Separately, a Phase 2 pilot study in *Becker muscular dystrophy (BMD)* is evaluating the safety, tolerability and exploratory clinical efficacy on motor function outcome.

Santhera is committed to fully realizing AGAMREE's potential in the treatment of DMD by investing in its market potential and refining its clinical development priorities for potential new indications. While the Company initially planned to provide more details on the development of a *second indication* in Q4 this year, Catalyst Pharmaceuticals, the partner in North America, will begin further clinical exploratory and development studies in 2025 to inform the strategy for future indications thereafter.

FINANCIAL PERFORMANCE

Half-year results

- Revenue from contracts with customers of CHF 14.1 million (H1-2023: CHF 3.9 million)
- Operating result of CHF -17.7 million (H1-2023: CHF -20.3 million)
- Net result of CHF -15.3 million (H1-2023: CHF -23.3 million)
- Cash flow from operating activities of CHF-15.3 million (H1-2023: CHF -15.4 million)
- Cash and cash equivalents of CHF 16.5 million (Dec 31, 2023: CHF 30.4 million)

Subsequent events and financing outlook

- Close of financings with Highbridge and R-Bridge (August 12, 2024) provided initial receipt of CHF 58 million net of transaction costs with future sales milestones of up to USD 8 million
- Repayment of maturing listed convertible bonds (CHF 14 million including interest)
- Extension of CHF 7 million private convertible bond until August 2025
- Cash reach into 2026 when cash flow break-even is expected

Net Revenue

In the first half-year 2024, Santhera reported revenue from contracts with customers of CHF 14.1 million (H1-2023: CHF 3.9 million). Net sales amounted to CHF 6.5 million following the launch of AGAMREE in Germany and Austria (H1-2023: CHF 1.0 million arising from RAXONE which ceased following the disposal

during H2-2023). Additionally, Santhera recognized CHF 7.7 million (H1-2023: CHF 3.0 million) from partners in China and North America reflecting milestones and product supply.

Cost of goods sold

Cost of goods sold amounted to CHF 5.2 million and increased on the prior year level (H1-2023: CHF 1.9 million), reflecting initiation of commercial supply and the amortization of intangible assets. Cost of goods for the six months includes non-cash intangible amortization of CHF 2.5 million (H1-2023: CHF 1.5 million), royalties payable of CHF 1.0 million (H1-2023: nil) as well as early-stage logistics and CMC set up expenses.

Operating expenses and result

Operating expenses of CHF 26.7 million (H1-2023: CHF 22.5 million) were 20% higher year-on-year, primarily due to an increase in activities to support the commercialization of AGAMREE.

Development expenses amounted to CHF 13.8 million (H1-2023: CHF 9.7 million). The increase of 40% arises from additional longer-term studies and CMC (chemistry, manufacturing, and controls) development activities to further enhance the commercial success of AGAMREE.

Marketing and sales expenses were CHF 4.7 million (H1-2023: CHF 4.3 million). This represents a slight increase due to higher activities for AGAMREE in Europe to support launches offset by reduction in expenses incurred in H1-2023 to support pre partnering US activities.

General and administrative expenses amounted to CHF 8.3 million (H1-2023: CHF 8.4 million), with activities focused in 2024 on supporting commercial growth.

The operating result amounted to a loss of CHF 17.7 million (H1-2023: loss of CHF 20.3 million).

Financial income and expenses

The financial income amounted to CHF 8.6 million (H1-2023: CHF 5.7 million). The increase was predominantly related to net positive changes in fair value of financial instruments, interest receivable and in (un)realized foreign exchange gains.

Financial expenses were CHF 6.0 million (H1-2023: CHF 8.8 million), primarily driven by interest payable and in (un)realized foreign exchange losses.

In summary, this resulted in a net financial income of CHF 2.7 million, compared with a net expense of CHF 3.1 million for H1-2023.

Net result

The net result in 2024 was a loss of CHF 15.3 million, compared to a net loss of CHF 23.3 million for H1-2023.

Cash balance and cash flows

As of June 30, 2024, the Company had cash and cash equivalents of CHF 16.5 million compared to CHF 30.4 million as of December 31, 2023, a decrease of CHF 13.9 million (H1-2023 an increase of CHF 0.3 million)

Net cash flow used in operating activities amounted to CHF 15.3 million (H1-2023: net cash outflow of CHF 15.4 million).

There was negligible cash flow from investing activities (H1-2023 income of CHF 5.7 million from proceeds of sale of shares).

Net cash flow used in/from financing activities was CHF -0.4 million (H1-2023: CHF 10.0 million). There were no financing inflows during the period due to the available cash balances, while additional finance was raised following the period end.

Assets and liabilities

Intangible assets decreased by CHF 2.5 million to CHF 71.5 million reflecting amortization in the period.

Total assets decreased by CHF 1.4 million to CHF 108.2 million as a result of a reduction in cash of CHF 13.9 million, offset by an increase in inventory and trade receivables following initial commercialization and milestones receivable.

Total liabilities increased by CHF 10.5 million to CHF 60.2 million mainly due to increase in trade payables relating to inventory purchases.

Shareholders' equity

Total consolidated equity as of June 30, 2024, amounted to CHF 48.1 million compared to CHF 59.9 million as of December 31, 2023.

Financing activities

As previously announced, additional funding was required to support ongoing activities and service debt obligations. In August, Santhera closed two financing agreements that provided the Company with gross funding totaling approximately CHF 69 million.

CHF 35 million received from Highbridge under a new term loan agreement

Santhera received CHF 35 million from a term loan financing from certain funds managed by Highbridge Capital Management, LLC (Highbridge). The loan has a four-year maturity with amortization in the amount of 15% per year, commencing after 24 months, and will pay a cash interest of 3-month SARON (floor of 2%) plus 9.75% per year. The transaction includes changes to the existing Highbridge private convertible bonds, extending CHF 7 million with a strike price of CHF 10 by 12 months to August 2025, and converting CHF 4 million, with a strike price of CHF 5 as well as issuing the new warrants to Highbridge.

USD 30 million received from R-Bridge for partial and capped royalty monetization

Upon closing of the royalty monetization financing agreement, R-Bridge paid an upfront of USD 30 million to Santhera and will make staged sales-related milestone payments that, if achieved, would result in total payments to Santhera of a further USD 8 million.

The royalty agreement with R-Bridge is partial and capped. Santhera is monetizing 75% of the future royalty income streams (net of any agreed payment obligations of Santhera to ReveraGen and Idorsia) from its licensing agreements for AGAMREE with Catalyst Pharmaceuticals, Inc. and with Sperogenix Therapeutics Ltd., in respect of net product sales occurring from July 1, 2024. Once the agreed threshold or duration of royalty payments is met, the North America and China royalty payments will revert back to Santhera. In addition, Santhera retained certain rights to buy back the royalty income stream.

Following closing of the financings, the proforma cash balance was CHF 72 million (August 13, 2024) after receipt of CHF 58 million net of transaction fees. After repayment of the maturing listed convertible bonds and interest in the amount of CHF 14 million, the remaining cash balance is expected to provide for a cash runway into 2026 and to expected cash flow break-even.

2024 Half-year Financial Information

Interim condensed consolidated income statement (for the six months ended June 30, in CHF thousands, except per share data)	**H1-2024** **(unaudited)**	H1-2023 (unaudited)
Net sales	**6,464**	969
Revenue from out-licensing transactions	**6,361**	1,921
Net sales to licensing partner	**1,289**	1,049
Revenue from contracts with customers	**14,114**	3,939
Cost of goods sold	**(5,215)**	(1,928)
of which amortization intangible assets	***(2,487)***	*(1,519)*
Development	**(13,771)**	(9,748)
Marketing and sales	**(4,660)**	(4,257)
General and administrative, other	**(8,276)**	(8,452)
Operating expenses	**(26,707)**	(22,457)
Operating result	**(17,741)**	(20,305)
Financial result, net	**2,652**	(3,115)
Income tax (expense)/benefit	**174**	84
Net result	**(15,263)**	(23,336)
Basic and diluted loss per share (in CHF)	**(1.35)**	(2.09)

Interim condensed consolidated balance sheet (in CHF thousands)	**Jun 30, 2024** **(unaudited)**	Dec 31, 2023 (audited)
Cash and cash equivalents	**16,491**	30,370
Other current assets	**17,149**	4,287
Noncurrent assets	**74,606**	74,972
Total assets	**108,246**	109,629
Equity	**48,057**	59,943
Noncurrent liabilities	**7,139**	5,371
Current liabilities	**53,050**	44,315
Total equity and liabilities	**108,246**	109,629

Interim condensed consolidated cash flow statement (for six months ended June 30, in CHF thousands)	**H1-2024** **(unaudited)**	H1-2023 (unaudited)
Net cash flow from/(used in) operating activities	**(15,282)**	(15,358)
Net cash flow from/(used in) investing activities	**10**	5,682
Net cash flow from/(used in) financing activities	**(358)**	9,979
Cash and cash equivalents at January 1	**30,370**	1,353
Cash and cash equivalents at June 30	**16,491**	1,674
Net increase/(decrease) in cash and cash equivalents	**(13,879)**	321

Share capital (number of shares with par value of CHF 0.10)	**Jun 30, 2024** **(unaudited)**	Dec 31, 2023 (audited)
Ordinary shares issued	**12,620,376**	12,620,376
Treasury shares	**918,814**	1,305,167
Conditional capital for employee participations (Art 3b)	**542,450**	542,450
Conditional capital for financing purposes (Art 3c)	**5,500,000**	5,500,000

Half-year Report

The Santhera Half-year Report 2024 is available for download on the Company's website at www.santhera.com/financial-reports.

Conference Call

Santhera will host a conference call on September 12, 2024, at 14:30 CEST / 13:30 BST / 08:30 EDT. CEO Dario Eklund, CFO Andrew Smith and CMO Shabir Hasham, MD, will discuss the 2024 half-year financial results and comment on ongoing corporate developments. Participants are invited to call one of the following numbers (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.

References

Publications and applicable drug labeling to which this press release makes reference to:
Labeling: United States Prescribing Information; European Union Summary of Product Characteristics
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Guglieri M et al (2022). JAMA Neurol. 2022;79(10):1005-1014. doi:10.1001/jamaneurol.2022.2480. Link.
Liu X et al (2020). Proc Natl Acad Sci USA 117:24285-24293
Heier CR et al (2019). Life Science Alliance DOI: 10.26508
Ward et al., WMS 2022, FP.27 - Poster 71. Link.
Hasham et al., MDA 2022 Poster presentation. Link.

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. The Company has an exclusive license from ReveraGen for all indications worldwide to AGAMREE® (vamorolone), a dissociative steroid with novel mode of action, which was investigated in a pivotal study in patients with Duchenne muscular dystrophy (DMD) as an alternative to standard corticosteroids. AGAMREE for the treatment of DMD is approved in the U.S. by the Food and Drug Administration (FDA), in the EU by the European Medicines Agency (EMA), and in the UK by the Medicines and Healthcare products Regulatory Agency (MHRA). Santhera has out-licensed rights to AGAMREE for North America to Catalyst Pharmaceuticals and for China to Sperogenix Therapeutics. For further information, please visit www.santhera.com.

AGAMREE® is a trademark of Santhera Pharmaceuticals.

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