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Ad hoc announcement pursuant to Art. 53 LR

Santhera Announces Closing of Exclusive North America License Agreement with Catalyst Pharmaceuticals for Vamorolone

Pratteln, Switzerland, July 19, 2023 – Santhera Pharmaceuticals (SIX: SANN) announces the closing of the exclusive license agreement for vamorolone in North America (NA) with Catalyst Pharmaceuticals, Inc. (NASDAQ: CPRX), announced on June 20, 2023 [1]. The proceeds from the agreement extend Santhera's cash reach into 2025. Together with the expected income from ongoing operations, it provides funding for the pre-commercialization and launch of vamorolone in Europe and enables the repayment of debt to Highbridge Capital, thereby also strengthening the Company's balance sheet.

The license agreement grants Catalyst commercialization rights in North America (NA) for vamorolone in Duchenne muscular dystrophy (DMD) and all potential future indications for a total consideration to Santhera of up to USD 231 million plus royalty payments from product sales.

"The partnership with Catalyst allows us to execute our European strategy for vamorolone, supported by adequate funding, and provides financial security for the Company's operations into 2025. It also, subject to regulatory approval, opens up the possibility of expanding vamorolone's potential by jointly addressing additional indications beyond DMD," said **Dario Eklund, Chief Executive Officer of Santhera**. "With partners in North America and China, it enables us to fully focus on bringing vamorolone to DMD patients in Europe, and we are preparing for a first launch in Germany which could occur as early as in late 2023, subject to approval. For markets outside the five largest European countries plus Benelux, where we will commercialize vamorolone ourselves, we continue to evaluate additional partnerships."

Upon closing of the agreement, Santhera will receive a USD 75 million upfront cash milestone payment from Catalyst and an additional USD 15 million through the sale of treasury shares to Catalyst. Subject to U.S. FDA approval of vamorolone in DMD, a decision expected by the October 26, 2023 PDUFA date, Santhera will receive another USD 10 million from Catalyst. Furthermore, Santhera is eligible to receive sales-based milestones of up to USD 105 million as well as royalties on sales. Catalyst will also pay USD 26 million of Santhera's third-party obligations at FDA approval, and royalty obligations on vamorolone sales in all indications in NA.

Debt repayment strengthens balance sheet and reduces future share dilution

The net receipts from the upfront cash milestone and the equity investment amount to CHF 78.6 million after transaction costs. Thereof, CHF 29 million will be used to fully repay current exchangeable notes to Highbridge Capital, significantly strengthening the Company's balance sheet. Also, as a result of settlement of the exchangeable notes, the underlying 3.9 million shares (as of June 30, 2023, and adjusted for the reverse share split) no longer need to be earmarked resulting in less future dilution than if the notes had been converted. Additionally, the first lien security and covenant obligations under the exchangeable note facility will be removed. An overview of Santhera's share capital after repayment to Highbridge and the share acquisition by Catalyst is provided here.

Proceeds secure implementation of European commercial strategy for vamorolone

The remaining cash and cash equivalents of CHF 49.6 million, together with expected milestone payments from partners and initial revenue proceeds in Europe, are expected to fund Santhera's current operating plan into early 2025. Importantly, they enable the Company to advance its commercialization strategy in Europe where Santhera plans to make vamorolone available to patients in key geographies including Germany, France, UK, Italy, Spain, and Benelux. Market access, the build-up of a core organization and stakeholder engagement activities in these priority countries are ongoing. Additionally, the early access programs applied for in France and the UK could, if granted, allow treatment of the first DMD patients with vamorolone in late fall of this year. Santhera expects a decision from the European Medicines Agency (EMA) in late 2023 and, subject to approval, plans to launch vamorolone in DMD in Germany as the first market, followed by a gradual rollout across the chosen key markets from 2024. The Company currently estimates it will reach annual sales in Europe in excess of EUR 150 million within the next five years in DMD alone, the first indication for vamorolone.

Outside the core European countries where Santhera will commercialize vamorolone directly, the Company seeks partnerships and has granted Catalyst a right of first negotiation in partnering discussions.

Closing of the transaction was subject to customary closing conditions and expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act.

About Vamorolone

Vamorolone is an investigational drug candidate with a mode of action based on binding to the same receptor as glucocorticoids but modifying its downstream activity and as such is considered a dissociative anti-inflammatory drug [3-6]. This mechanism has shown the potential to 'dissociate' efficacy from steroid safety concerns and therefore vamorolone could emerge as an alternative to existing corticosteroids, the current standard of care in children and adolescent subjects with DMD. In the pivotal VISION-DMD study, vamorolone met the primary endpoint Time to Stand (TTSTAND) velocity versus placebo (p=0.002) at 24 weeks of treatment and showed a good safety and tolerability profile [2]. The most commonly reported adverse events versus placebo from the VISION-DMD study were cushingoid features, vomiting and vitamin D deficiency. Adverse events were generally of mild to moderate severity.

Vamorolone has been granted Orphan Drug status for DMD in the U.S. and in Europe and has received Fast Track and Rare Pediatric Disease designations by the U.S. FDA and Promising Innovative Medicine (PIM) status from the UK MHRA for DMD. Vamorolone is an investigational medicine and is currently not approved for use by any health authority.

References:

[1] Press release "Santhera Grants Exclusive North America License for Vamorolone to Catalyst Pharmaceuticals in Deal Valued at up to USD 231 Million Plus Royalties", June 20, 2023. Link.
[2] Guglieri M et al (2022). JAMA Neurol. 2022;79(10):1005-1014. doi:10.1001/jamaneurol.2022.2480. Link.
[3] Mah JK et al (2022). JAMA Netw Open. 2022;5(1):e2144178. doi:10.1001/jamanetworkopen.2021.44178. Link.
[4] Guglieri M et al (2022) JAMA. doi:10.1001/jama.2022.4315
[5] Heier CR et al (2019). Life Science Alliance DOI: 10.26508
[6] Liu X et al (2020). Proc Natl Acad Sci USA 117:24285-24293

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 for all indications worldwide to 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. For vamorolone in the treatment of DMD, Santhera has a new drug application (NDA) under review by the U.S. FDA, a marketing authorization application (MAA) under review by the European Medicines Agency (EMA) and an MAA submitted to the UK Medicines and Healthcare products Regulatory Agency (MHRA). Santhera has out-licensed rights to vamorolone for North America to Catalyst Pharmaceuticals and for China to Sperogenix Therapeutics. 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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