

## **Santhera Exercises Option to Obtain Worldwide Rights to Vamorolone in Duchenne Muscular Dystrophy and All Other Indications**

- *License gives Santhera worldwide rights to vamorolone, now also including the major markets Japan and South Korea, and paves the way for partnering in additional indications*
- *Agreements with Idorsia and ReveraGen give Santhera immediate control over vamorolone and defer milestone-related payments*
- *Transaction establishes Santhera as a leading company in rare neuromuscular diseases with two late-stage assets addressing the medical need of DMD patients from early to late disease stages*

**Pratteln, Switzerland, and Rockville, MD, USA, September 2, 2020 – Santhera Pharmaceuticals (SIX: SANN) announces that it has signed agreements with Idorsia (SIX: IDIA) and ReveraGen BioPharma Inc., making Santhera a direct license holder of vamorolone. Under the agreements, Santhera has obtained an exclusive license from ReveraGen, the originator of vamorolone, for all indications worldwide. The agreements create further value for Santhera through the transfer of rights for the previously excluded markets Japan and South Korea, the right to grant sublicenses and a share in the expected Priority Review Voucher. Vamorolone, a first-in-class anti-inflammatory drug candidate with a novel mode of action, is currently being investigated in the pivotal Phase 2b VISION-DMD study in patients with Duchenne muscular dystrophy (DMD) by originator ReveraGen and completion of study enrollment is expected shortly.**

Under the amended terms, Santhera expects a reduction in cash outflow in the range of USD 18-24 million in the next 12-18 months. In exchange for the revised license rights, Idorsia will receive 366,667 Santhera shares and an exchangeable note in the amount of CHF 10 million and ReveraGen will receive USD 7 million, in monthly instalments of up to USD 500,000, to fund the ongoing clinical development of vamorolone.

**Dario Eklund, Chief Executive Officer of Santhera,** said: “We are excited about the license transfer of vamorolone to Santhera. Our decision to exercise the option now has been driven by a combination of factors including the availability of encouraging clinical efficacy and safety data with vamorolone, enhanced deal terms and the ability to gain full control over the asset. We look forward to contributing our significant expertise to advancing vamorolone in DMD and exploring additional business development opportunities. We believe that having two promising, complementary, late stage assets for DMD in our pipeline will enable increased access to potentially transformative treatments for a wider patient population. We are grateful to Idorsia, our anchor shareholder, for enabling early access to the license, highlighting its confidence in Santhera as the best suited company to bring vamorolone to patients.”

**Eric Hoffman, PhD, Vice President of Research of ReveraGen BioPharma,** said: “We are delighted about the revised contractual arrangement and being able to work directly with Santhera as the licensee for vamorolone. Santhera’s experience in both development of DMD drug candidates and the commercialization of a rare disease product positions it well to bring vamorolone to patients. Our work to date clearly shows that vamorolone not only holds the potential to become a new standard of care for patients with DMD but also could benefit patients in a number of other inflammatory diseases.”

**The agreements now signed with ReveraGen and Idorsia**

Idorsia has assigned its original agreement with ReveraGen to Santhera. Santhera has thus become a direct contracting party with ReveraGen and with a signed early option exercise this allows Santhera to gain exclusive and immediate access to vamorolone and defers some early milestone-related payments until after study readout. Under the terms of the agreements now signed with ReveraGen and Idorsia, Santhera has obtained an exclusive license, including sublicensing rights, for vamorolone in all indications and all territories worldwide, now also including Japan and South Korea. Additionally, ReveraGen is the holder of a Rare Pediatric Disease designation, which may result in receipt of a Priority Review Voucher upon approval of vamorolone for DMD. Santhera will have a share in any revenues of a potential sale of such a voucher.

The license agreements [1] were revised and include the following term amendments:

As consideration for the assignment of its licensing option for vamorolone to Santhera, Idorsia will receive 366,667 Santhera shares, increasing Idorsia's equity position in Santhera to close to 12%. Milestone payments by Santhera up to and including potential FDA approval will be reduced by USD 18 million to USD 72 million (previously USD 90 million). Santhera's obligations are a payment of up to USD 7 million, payable in monthly instalments of up to USD 500,000 to ReveraGen, to fund development including the Phase 2b VISION-DMD study; USD 5 million to ReveraGen at the time when FDA supports an NDA filing with Phase 2b 6-month data; a non-interest bearing exchangeable note to Idorsia in the amount of CHF 10 million; and USD 50 million (previously USD 60 million) in total for FDA approval. Since the exchangeable note is payable up to 65% in Santhera shares, at Santhera's discretion, this could potentially reduce cash outlay by an additional USD 6.5 million. Furthermore, Santhera will receive 10% of any potential proceeds that could arise from the monetization of the expected Priority Review Voucher. Upon achievement of the first USD 100 million revenue, an additional USD 5 million milestone payment is due to Idorsia.

Santhera estimates the peak sales potential for vamorolone for the DMD indication alone to be in excess of USD 500 million in the US and the largest five EU countries.

**With Puldysa® and vamorolone, Santhera is building a complementary DMD product portfolio**

Vamorolone is in development for young DMD patients requiring an anti-inflammatory, muscle strengthening treatment before onset of respiratory function decline. Based on the cumulative knowledge obtained from extensive non-clinical studies and Phase 1 and Phase 2a clinical studies with vamorolone, ReveraGen is currently conducting the pivotal Phase 2b VISION-DMD trial and anticipates full study enrollment shortly. Subject to positive results of the first 6-month treatment period, now expected in the second quarter of 2021 due to delays caused by the Covid-19 pandemic, this would pave the way for a regulatory submission to the US FDA in the fourth quarter of 2021.

Puldysa (idebenone) for patients with DMD in respiratory function decline who are not taking glucocorticoids is currently under regulatory review in Europe for which Santhera anticipates a CHMP opinion in the fourth quarter of 2020. The Company expects the combination of vamorolone and Puldysa to address the medical needs of DMD patients, from early to late disease stages, irrespective of age, underlying dystrophin mutation or ambulatory status.

Vamorolone and Puldysa have been granted Orphan Drug status in the US and in Europe, Fast Track and Rare Pediatric Disease designations by the US FDA and Promising Innovative Medicine (PIM) status by the UK MHRA. In the UK, Puldysa is available to patients through the Early Access to Medicines Scheme (EAMS).

*Santhera will further discuss the option exercise in the conference call on the occasion of the publication of the First Half-year Results on September 8, 2020.*

Reference:

[1] Press release “Santhera Enters into Agreement to Acquire Option from Idorsia for Exclusive Sub-License of First-in-class Dissociative Steroid Vamorolone”, November 20, 2018, accessible [here](#).

**About Vamorolone – a first-in-class anti-inflammatory drug candidate with a novel mode of action**

Vamorolone is a first-in-class drug candidate that binds to the same receptors as corticosteroids but modifies the downstream activity of the receptors. This has the potential to ‘dissociate’ efficacy from typical steroid safety concerns and therefore could emerge as a valuable alternative to corticosteroids, the current standard of care in children and adolescent patients with DMD. There is a clear unmet medical need in this patient group as high dose corticosteroids have significant systemic side effects that detract from patient quality of life. Vamorolone is being developed by US-based ReveraGen BioPharma Inc. with participation in funding and design of studies by several international non-profit foundations, the US National Institutes of Health, the US Department of Defense and the European Commission’s Horizon 2020 program.

**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 is building a Duchenne muscular dystrophy (DMD) product portfolio to treat patients from early to late disease stages, irrespective of causative mutations, ambulatory status or age. A marketing authorization application for Puldysa® (idebenone) is currently under review by the European Medicines Agency. Santhera has an exclusive license for all indications worldwide to vamorolone, a first-in-class anti-inflammatory drug candidate with novel mode of action, currently investigated in a pivotal study in patients with DMD as an alternative to standard corticosteroids. The clinical stage pipeline also includes lonodelestat (POL6014) to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases, as well as omigapil and an exploratory gene therapy approach targeting congenital muscular dystrophies. Santhera out-licensed ex-North American rights to its first approved product, Raxone® (idebenone), for the treatment of Leber's hereditary optic neuropathy (LHON) to Chiesi Group. Further information at [www.santhera.com](http://www.santhera.com).

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

**About ReveraGen BioPharma**

ReveraGen was founded in 2008 to develop first-in-class dissociative steroidal drugs for Duchenne muscular dystrophy and other chronic inflammatory disorders. The development of ReveraGen’s lead compound, vamorolone, has been supported through partnerships with foundations worldwide, including Muscular Dystrophy Association USA, Parent Project Muscular Dystrophy, Foundation to Eradicate Duchenne, Save Our Sons, JoiningJack, Action Duchenne, CureDuchenne, Ryan’s Quest, Alex’s Wish, DuchenneUK, Pietro’s Fight, Michael’s Cause, and Duchenne Research Fund. ReveraGen has also

received generous support from the US Department of Defense CDMRP, National Institutes of Health (NCATS, NINDS, NIAMS), and European Commission (Horizon 2020). [www.reveragen.com](http://www.reveragen.com)

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