

Santhera will hold a webcast tomorrow, November 21, 2018 at 13:00 CET, 12:00 GMT, 07:00 EST. Details at the end of statement.

Santhera Enters into Agreement to Acquire Option from Idorsia for Exclusive Sub-License of First-in-class Dissociative Steroid Vamorolone

- *Vamorolone in clinical development for Duchenne muscular dystrophy (DMD) by ReveraGen BioPharma Inc. – pivotal VISION-DMD Phase IIb study ongoing*
- *Vamorolone has the potential to become standard of care in young patients with DMD*
- *Positions Santhera as a leading company in the DMD space with two late-stage assets addressing the medical need of DMD patients at all disease stages*
- *Idorsia to become the largest shareholder in Santhera with a 13.3% equity stake*

Pratteln and Allschwil, Switzerland, November 20, 2018 – Santhera Pharmaceuticals (SIX: SANN) and Idorsia Ltd (SIX: IDIA) have entered into an agreement under which Santhera will acquire the option to exclusively in-license, by way of sub-license, the first-in-class dissociative steroid vamorolone in all indications and all countries worldwide except Japan and South Korea. Initial clinical data suggest that vamorolone has the anti-inflammatory efficacy of steroids with reduced steroid-associated safety concerns, which would represent a significant improvement over current standard of care glucocorticoid therapy in patients with Duchenne muscular dystrophy (DMD), vamorolone’s lead indication.

Thomas Meier, PhD, Chief Executive Officer of Santhera, said: “Vamorolone is a highly promising drug candidate for the treatment of patients with DMD and a perfect strategic fit alongside idebenone. Our late-stage DMD drug portfolio covers a broad DMD patient spectrum, irrespective of genetic background, disease stage or age. This agreement underscores our strategy of in-licensing high-quality, late-stage rare disease assets, which leverage our existing capabilities and expertise. We are also delighted to welcome Idorsia as our largest shareholder and partner and look forward to working with ReveraGen in the development of vamorolone, which has the potential to replace standard glucocorticoids as treatment for DMD.”

Vamorolone – first-in-class dissociative steroid

Vamorolone is a first-in-class drug candidate that binds to the same receptors as glucocorticoids but modifies the downstream activity of the receptors. This has the potential to ‘dissociate’ efficacy from typical steroid safety concerns and therefore could replace existing glucocorticoids, the current standard of care in children and adolescent patients with DMD. There is significant unmet medical need in this patient group as high dose glucocorticoids have severe systemic side effects, which limit long-term usage.

Vamorolone was discovered by US-based ReveraGen BioPharma Inc. and has been developed with participation in funding and design of studies by 12 international non-profit foundations, the US National Institutes of Health, the US Department of Defense and the European Commission’s Horizon 2020 program. Actelion had acquired an option to license the product in 2016. This option was subsequently transferred to Idorsia following the acquisition of Actelion by Johnson & Johnson in 2017.

Eric Hoffman, PhD, Chief Executive Officer of ReveraGen, commented: “Our hope for vamorolone is that it can replace existing glucocorticoids in DMD therapy. Early clinical development of vamorolone in patients with DMD, using an innovative approach with an array of pre-selected biomarkers in multiple contexts of use, suggests that vamorolone preserves anti-inflammatory efficacy while decreasing steroid-associated safety concerns. I am delighted to work with Santhera to advance this exciting therapeutic candidate for patients with DMD.”

Vamorolone in DMD

Following single and multiple ascending dose clinical pharmacology studies (VBP15-001) in healthy volunteers [1] vamorolone completed a Phase IIa study (VBP15-002) in 48 boys with DMD aged 4 to <7 years. Vamorolone was reported to be safe and well tolerated up to 6.0mg/kg/day, around 10 times the standard glucocorticoid dose [2]. A 6-month extension study (VBP15-003) also demonstrated dose-dependent improvement in timed function tests which was comparable to standard glucocorticoid treatment.

The ongoing Phase IIb VISION-DMD study (VBP15-004) builds on the available promising preliminary safety and efficacy data from Phase IIa and is designed to bridge exploratory biomarker data to clinical outcomes. This pivotal study will enroll approximately 120 boys aged 4 to <7 with DMD that have not yet been treated with glucocorticoids, randomized to one of four groups: low dose vamorolone (2 mg/kg/day), high dose vamorolone (6 mg/kg/day), prednisone (0.75 mg/kg/day), or placebo. After the initial 24-week treatment period, the prednisone and placebo groups will cross-over to low dose or high dose vamorolone. The second treatment period then has all patients treated for an additional 20 weeks with vamorolone. Clinical outcomes for efficacy include timed function tests and measures of muscle strength and endurance. Clinical outcomes for safety include monitoring of bone changes, weight changes, cataracts, and biomarkers of metabolic disturbances.

The study is being conducted at approximately 30 sites across North America, Europe, Israel and Australia. Enrollment, which began in August 2018, is expected to take about 12 months, with a total study duration of about 24 months. If successful, the data filing with health authorities in the US is anticipated by the end of 2020 and in the EU in 2021. Vamorolone has received Orphan Drug Designation in the US and in Europe and fast-track status in the US.

Santhera management anticipates peak sales potential for vamorolone for the DMD indication of USD 500 million.

The Agreement

Under the terms of the agreement, Idorsia will grant Santhera the option to obtain an exclusive sub-license for vamorolone in all indications and all territories except Japan and South Korea. Idorsia will receive as consideration for entering into the agreement 1,000,000 (one million) new registered shares from Santhera’s existing authorized share capital and an upfront cash component of USD 20 million, of which USD 15 million is intended to compensate Idorsia for its investment into the Phase IIb VISION-DMD study currently conducted by ReveraGen. While the cash component of the consideration is subject to financing, the share component of the consideration is unconditional and, like the cash component, not redeemable under any circumstances. As a consequence of the transaction, Idorsia will become the largest shareholder in Santhera with a 13.3% equity position. The shares to be issued to Idorsia will be

subject to a lock-up undertaking expiring if and when vamorolone receives marketing authorization in DMD in the United States. Santhera may exercise the option upon receipt of data from the Phase IIb VISION-DMD study (VBP15-004) and following a one-time consideration to Idorsia of USD 30 million.

Following the exercise of the worldwide vamorolone license option by Idorsia and exercise of the vamorolone sub-license option for all territories worldwide except Japan and South Korea by Santhera, Santhera will pay to Idorsia regulatory and commercial milestone payments of up to USD 80 million in the DMD indication and four one-time sales milestone payments of up to USD 130 million in aggregate. Regulatory milestone payments by Santhera to Idorsia for three additional indications amount to up to USD 205 million in aggregate. Upon commercialization of vamorolone, Santhera has committed to pay tiered royalties ranging from a single-digit percentage to low double-digit percentage on the annual net sales of vamorolone to Idorsia.

Jean-Paul Clozel, MD, Chief Executive Officer of Idorsia, concluded: “With four compounds in late-stage clinical development and more innovative compounds coming through the pipeline, Idorsia’s newly established commercial function has many assets to focus on. We have decided to hand the option to license vamorolone to Santhera because they are ideally placed to maximize the potential of this asset. If successful, Santhera’s network and expertise in the field of DMD will allow patients to benefit from this potential new treatment approach as soon as possible. In addition, with this agreement we become Santhera’s largest shareholder, so we remain highly motivated and committed to make vamorolone a success.”

Centerview Partners acted as exclusive strategic and financial advisor to Santhera for this transaction.

Santhera Webcast & Conference Call

Santhera will hold an audio webcast / conference call tomorrow, November 21, 2018 at 13:00 CET, 12:00 GMT, 07:00 EST to discuss the agreement on vamorolone. Participants are invited to join either the audio webcast or telephone conference 10-15 minutes before the start:

Webcast: click [this link](#) to access the webcast

Telephone conference: dial one of the following numbers (no code required):

Europe: +41 58 310 50 00

UK: +44 207 107 0613

USA: +1 631 570 5613

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Notes to the editor

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.

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 (Horizons 2020). www.reveragen.com

About Idorsia

Idorsia Ltd is reaching out for more - We have more ideas, we see more opportunities and we want to help more patients. In order to achieve this, we will develop Idorsia into one of Europe's leading biopharmaceutical companies, with a strong scientific core.

Headquartered in Switzerland - a biotech-hub of Europe - Idorsia is specialized in the discovery and development of small molecules, to transform the horizon of therapeutic options. Idorsia has a broad portfolio of innovative drugs in the pipeline, an experienced team, a fully-functional research center, and a strong balance sheet - the ideal constellation to bringing R&D efforts to business success.

Idorsia was listed on the SIX Swiss Exchange (ticker symbol: IDIA) in June 2017 and has over 700 highly qualified professionals dedicated to realizing our ambitious targets.

About the exclusive worldwide license agreement between Idorsia and ReveraGen

Idorsia's agreement with ReveraGen relating to the vamorolone license option was last renewed and amended on November 5, 2018. Under the agreement, as amended, Idorsia is entitled to exercise an option to obtain the exclusive worldwide license rights relating to vamorolone at any time, but not later than upon receipt of the Phase IIb study results in exchange for a consideration of USD 20 million. If the option is exercised, ReveraGen will be entitled to regulatory and commercial milestone payments from Idorsia of up to USD 75 million in the DMD indication and three one-time sales milestone payments of up to USD 120 million in aggregate. Regulatory milestone payments by Idorsia to ReveraGen for three additional indications amount to up to USD 190 million in aggregate. Upon commercialization of vamorolone, Idorsia has committed to pay tiered single-digit to low double-digit royalties on the annual net sales of vamorolone to ReveraGen.

References

[1] Hoffman E et al. (2018). Phase 1 trial of vamorolone, a first-in-class steroid, shows improvements in side effects via biomarkers bridged to clinical outcomes. *Steroids* 134: 43-52

[2] Conklin LS et al. (2018). Phase IIa trial in Duchenne muscular dystrophy shows vamorolone is a first in-class dissociative steroidal anti-inflammatory drug. *Pharmacological Research* 136:140-150.

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