

## Santhera to acquire exclusive sub-license of Vamorolone

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**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**



Santhera Pharmaceuticals which is Swiss specialty pharmaceutical company focused on the development and commercialization of innovative medicines for rare and other diseases and Idorsia Ltd which is specialized in the discovery and development of small molecules, to transform the horizon of therapeutic options 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, 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."

Discovered by US-based ReveraGen BioPharma Inc., 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.

Eric Hoffman, 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."

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 2b 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 2b 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, 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."