Ad hoc announcement pursuant to Art. 53 LR

Santhera and ReveraGen Announce Successful FDA Pre-NDA Meeting for Vamorolone in Duchenne Muscular Dystrophy

Pratteln, Switzerland, and Rockville, MD, USA, November 17, 2021 –Santhera Pharmaceuticals (SIX: SANN) and ReveraGen Biopharma (US: private) announce the successful completion of a first pre-NDA meeting with the U.S. Food and Drug Administration (FDA) for vamorolone for the treatment of Duchenne muscular dystrophy (DMD). The FDA considered both the proposed clinical efficacy and safety data sufficient for an NDA filing. Based on the Fast Track Designation for vamorolone, the FDA deemed the plan to pursue a rolling NDA review acceptable. The NDA submission is to commence in Q1-2022.

The purpose of the routine Type B pre-NDA meeting was to obtain agreement from the FDA on the sufficiency and adequacy of the clinical data to support an NDA filing of vamorolone for the treatment of DMD. In its conclusions from this meeting, the FDA agreed that (i) the efficacy of vamorolone as demonstrated in the 24-week phase of the pivotal VISION-DMD study supports an NDA application and that (ii) the results from the 24-week double-blind VISION-DMD study phase and the open-label studies provide sufficient safety data to support an NDA filing of vamorolone for the treatment of DMD. Acceptance of the NDA will be subject to FDA's review of the complete filing.

Based on the feedback from the FDA, Santhera and ReveraGen will initiate the rolling NDA submission in Q1-2022. As part of the process, additional pre-NDA meetings with the FDA are planned to address other sections of the filing. Vamorolone has been granted Orphan Drug status in the US for DMD and has received Fast Track and Rare Pediatric Orphan designations by the US FDA.

While outside of the scope of the pre-NDA meeting and with reference to a PPSR (Proposed Pediatric Study Request)-related question, the FDA also noted in its response that, as a synthetic steroid, vamorolone may have potential public health benefits for other indications. On this basis, Santhera will continue to collaborate with the FDA and evaluate opportunities for indications beyond DMD.

"We are very pleased with the positive outcome of this first pre-NDA meeting and look forward to initiating our NDA submission. As we are nearing important regulatory milestones on our journey to bring vamorolone to patients, we thank all participants, their families and caregivers, as well as investigators and study personnel, for their commitment to the vamorolone program," said **Eric Hoffman, PhD, President and CEO of ReveraGen BioPharma**.

"The feedback from the FDA on our submission plans is encouraging and an important confirmatory step of the progress we have made in advancing vamorolone as a foundational treatment option in DMD. We now look forward to working with the FDA and other regulatory authorities to bring vamorolone to market as soon as possible," said **Dario Eklund, Chief Executive Officer of Santhera**.

Prior to the meeting with the FDA, Santhera and ReveraGen submitted a data package which centered around the positive 24-week results from the pivotal Phase 2b VISION-DMD study in patients aged 4-<7 years. In this study, vamorolone showed statistically significant and clinically meaningful differences from placebo on the primary efficacy endpoint (time-to-stand/TTSTAND velocity), with consistently strong efficacy results across other pre-specified secondary and exploratory efficacy endpoints [1, 2]. The briefing package was supplemented with the open-label Phase 2a long-term treatment data that provide supportive information regarding dose-response, durability of treatment effect, and long-term safety over a 2.5-year follow up period [3]. Vamorolone treatment has been shown to preserve height trajectory and had a significantly lower adverse impact on measures of bone health and behavior changes compared to prednisone [4-7]. In clinical studies, vamorolone was generally safe and well tolerated. 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.

As previously announced, the FDA's support of an NDA submission for vamorolone based on the 24-week data from the VISION-DMD study triggers a milestone payment of USD 5 million to the licensor ReveraGen, and will enable Santhera to draw down CHF 5 million under the financing agreement with Highbridge Capital Management LLC.

Vamorolone was discovered by US-based ReveraGen BioPharma, Inc. and is being developed in collaboration with Santhera who owns worldwide rights to the drug candidate for all indications.

About Vamorolone

Vamorolone is a first-in-class drug candidate that binds to the same receptor as corticosteroids but modifies its downstream activity and as such is a dissociative agonist [8-10]. This mechanism has the potential to 'dissociate' efficacy from typical steroid safety concerns and therefore vamorolone could emerge as a promising alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD. Start of US NDA rolling submission for DMD is anticipated in Q1-2022. Vamorolone has been granted Orphan Drug status in the US and in Europe for DMD, and has received Fast Track and Rare Pediatric Disease designations by the US 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] ClinicalTrials.gov Identifier: NCT03439670

[2] Press release "Santhera and ReveraGen Announce Positive and Statistically Highly Significant Topline Results with Vamorolone in Pivotal VISION-DMD Study", June 1, 2021, link

[3] Press release "Santhera and ReveraGen Announce New 2.5-year Treatment Data with Vamorolone in Duchenne Muscular Dystrophy", April 28, 2021, link

[4] Hoffman E. Presented at virtual PPMD Annual Conference, June 22-26, 2021.

[5] Mah JK, et al. 2021. [Manuscript in progress]

[6] Hoffman E, et al. Neurology. 2019 Sep 24; 93(13): e1312–e1323

[7] Data on File. ReveraGen Biopharma, Rockville, MD

[8] Heier CR at al. (2013). EMBO Mol Med 5: 1569–1585.

[9] Reeves EKM, et al (2013). Bioorg Med Chem 21(8):2241-2249.

[10] 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. Santhera has an exclusive license for all indications worldwide to vamorolone, a first-in-class dissociative steroid with novel mode of action, which was investigated in a pivotal study in patients with DMD as an alternative to standard corticosteroids. The clinical stage pipeline also includes lonodelestat to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases as well as an exploratory gene therapy approach targeting congenital muscular dystrophies. 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.

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, Duchenne Research Fund, and Jesse's Journey. 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

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