Santhera and ReveraGen to Present New Data with Vamorolone at 2022 Parent Project Muscular Dystrophy Conference

Pratteln, Switzerland, and Rockville, MD, USA, June 9, 2022 – Santhera Pharmaceuticals (SIX: SANN) and ReveraGen BioPharma, Inc announce presentations of new data with vamorolone in Duchenne muscular dystrophy (DMD).

Cross-study comparisons of long-term efficacy, impact on growth velocity, and frequency and severity of spinal fractures for vamorolone versus current standard of care from the Phase 2b VISION-DMD study and the FOR-DMD study will be presented during the poster session at the 2022 Annual Conference of Parent Project Muscular Dystrophy (PPMD) to be held from June 23-26, 2022, in Scottsdale, AZ, USA.

"Data being presented by our academic collaborators at PPMD this year will provide further insight into our understanding of the potential of a dissociative steroid, such as vamorolone, in maintaining muscle strength whilst addressing important safety concerns such as preventing growth stunting and reducing the frequency and severity of bone related side effects such a spinal fractures in the treatment of Duchenne Muscular Dystrophy," said **Dr. Shabir Hasham, Chief Medical Officer and Head Global Medical Affairs of Santhera**.

Propensity score matched populations from the pivotal Phase 2b VISION-DMD study and the FOR-DMD study compared the efficacy of vamorolone to prednisone and deflazacort over 48-weeks. Safety analyses focused on the comparison of the long-term endpoints of height and spinal fractures, with vamorolone data collected in study VBP15-LTE (long-term extension) [1] versus prednisone and deflazacort data from the FOR-DMD study [2] over 2.5 years.

Abstracts will be available on the PPMD website.

Santhera will be participating in the PPMD Resource Fair (June 23-24) at the conference where medical representatives of the Company will be onsite to provide scientific information and latest updates.

Santhera expects to complete the rolling new drug application (NDA) filing for vamorolone in DMD to the U.S. FDA in June 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.

About Vamorolone

Vamorolone is a drug candidate with a novel mode of action that binds to the same receptor as corticosteroids but modifies its downstream activity and as such is a dissociative agonist [2-4]. 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 adolescents 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. In clinical studies, vamorolone was generally 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.

About the FOR-DMD Study

The Finding the Optimum Regimen for DMD (FOR-DMD; ClinicalTrials.gov identifier NCT01603407 / EudraCT 2010-023744-33) study is a randomized, prospective, multicenter, double-blind, comparative study of the efficacy and safety of 3 corticosteroid regimens (prednisone daily, prednisone intermittent or deflazacort daily) in boys aged ≥4 years and <8 years with a confirmed diagnosis of DMD and who were corticosteroid naïve at study entry.

References:
[1] Mah JK et al (2022). JAMA Netw Open. 2022;5(1):e2144178. doi:10.1001/jamanetworkopen.2021.44178
[2] Guglieri, et al (2022) JAMA. doi:10.1001/jama.2022.4315
[3] Heier CR at al (2019). Life Science Alliance DOI: 10.26508
[4] 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 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 Company plans to complete the rolling submission of its filing for approval for vamorolone with the U.S. FDA in June 2022. 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.

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 Defeat Duchenne Canada. 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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