# Santhera and ReveraGen Announce First Participant Dosed in FDA-funded Phase 2 Pilot Study with Vamorolone in Becker Muscular Dystrophy

**Pratteln, Switzerland, and Rockville, MD, USA, August 22, 2022 – Santhera Pharmaceuticals (SIX: SANN) and ReveraGen BioPharma, Inc announce that the first patient has been dosed in a Phase 2 pilot study to assess vamorolone in Becker muscular dystrophy (BMD), funded by the U.S. Food and Drug Administration (FDA).**

This Phase 2 pilot study is a randomized, double-blind, placebo-controlled study to evaluate the safety, tolerability and exploratory clinical efficacy on motor function outcomes of vamorolone compared to placebo over a treatment period of 24 weeks in 39 males (aged ≥18 and <65 years) with BMD (ClinicalTrials.gov id: NCT05166109). Participants will be randomized 2:1 to vamorolone 500 mg (250 mg for body weight <50 kg) daily or placebo. The clinical trial plans to enroll at sites in Pittsburgh (USA) and Padova (Italy).

The study is funded by a USD 1.2 million grant from the FDA under their "Clinical Studies of Orphan Products Addressing Unmet Needs of Rare Diseases (R01)" grants program. The grant adds to existing grants from the National Institutes of Health (NIH) - National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), and the Foundation to Eradicate Duchenne to carry out a clinical trial of vamorolone in adults with BMD.

"There are currently no approved drugs for BMD in any country, and no drugs in clinical development for this indication in the USA or Europe," said **Eric Hoffman, PhD, President and CEO of ReveraGen BioPharma**. "Vamorolone has shown efficacy in the pivotal VISION-DMD study in Duchenne muscular dystrophy (DMD), a more severe but related disease, and, based on these findings and its mechanisms of action, this developmental compound may show a benefit in BMD."

"The treatment of BMD is lacking standard of care recommendations albeit there is a high unmet medical need, and about one fifth of BMD patients use some chronic steroid dosing which is often not well tolerated," said **Shabir Hasham, MD, Chief Medical Officer and Head Global Medical Affairs of Santhera**. "Vamorolone could address some important safety concerns that may lead to poor tolerability or early treatment discontinuation, and thus may potentially represent a novel therapeutic approach to the treatment of BMD as a chronic therapy."

"Vamorolone has been shown to suppress dystrophin-targeted microRNAs, and thus has the potential to increase dystrophin levels in BMD muscle," said **Paula Clemens, MD, University of Pittsburgh School of Medicine**, and the Study Chair of the clinical trial.

BMD, the second most common progressive muscle wasting disease, is similar to DMD, but usually milder. It is caused by the same dystrophin gene (allelic) mutations as DMD but shows residual dystrophin protein in muscle, and variable onset and progression of muscle weakness. Additional rationale supporting potential benefits of vamorolone in BMD is based on studies in mouse models, where vamorolone increased dystrophin levels via suppression of inflammation-associated microRNA pathways. On this basis, vamorolone is anticipated to increase dystrophin levels in BMD patient muscle [1-3].

**About Vamorolone**

Vamorolone is a drug candidate with a mode of action that binds to the same receptor as corticosteroids but modifies its downstream activity and as such is considered a dissociative anti-inflammatory drug [4-7]. 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. 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.

Santhera expects to complete the rolling new drug application (NDA) filing for vamorolone in DMD to the U.S. FDA in Q4-2022. 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.

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**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 Q4-2022. 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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