Santhera and ReveraGen to Present Findings from Pivotal VISION-DMD Study with Vamorolone at Parent Project Muscular Dystrophy 2021 Conference

Pratteln, Switzerland, and Rockville, MD, USA, June 24, 2021 – Santhera Pharmaceuticals (SIX: SANN) and ReveraGen BioPharma, Inc (US: private) announce presentations of the positive and statistically highly significant topline results from the VISION-DMD study at the Parent Project Muscular Dystrophy (PPMD) 2021 Virtual Annual Conference (June 23-26, 2021).

Recently, Santhera and ReveraGen announced positive and statistically highly significant 24-week data from the pivotal Phase 2b VISION-DMD study in patients with Duchenne muscular dystrophy (DMD) [1, 2]. In this trial, vamorolone demonstrated efficacy versus placebo over a three-fold dose range of 2 to 6 mg/kg/day versus placebo (p=0.002) and showed for both doses a favorable safety and tolerability profile versus the active control arm of prednisone 0.75mg/kg/day. This unique benefit risk profile may represent a promising therapeutic approach for the chronic therapy of DMD patients, allowing for an individualized and optimized treatment regimen.

The already announced top-line results as well as additional safety analyses will be presented at PPMD's virtual conference (June 23-26, 2021) on the following occasions:

"In the Pipeline: Reducing inflammation" – Friday, June 25, 4:05 EDT
Presentation of the VISION-DMD 24-week results by Eric Hoffman, PhD, President and CEO at ReveraGen BioPharma, followed by Q&A.

"Recently Reported Updates: ReveraGen (partnership with Santhera)" -- Saturday, June 26, 3:00 EDT
Late-breaking session with presentation by Presentation by Eric Hoffman, PhD.

"Evolution of time to stand velocity in glucocorticoid-using and non-using patients with DMD"
Poster by Craig McDonald, MD, Professor and Chair, Department of Physical Medicine & Rehabilitation and Director of Neuromuscular Disease Clinics, UC Davis Health, USA, and his collaborators can be viewed in the online poster session.

"Duchenne Drug Development Update – Vamorolone in Duchenne muscular dystrophy"
Santhera's company update on vamorolone and DMD presented by Jodi Wolff, PhD, Head of Global Patient Advocacy, will be available on PPMD's website.

Vamorolone is being investigated as a first-in-class dissociative steroid with lower incidence of corticosteroid-associated adverse effects. The pivotal VISION-DMD study met its primary endpoint of superiority in change of time to stand from supine positioning to standing (TTSTAND) velocity with vamorolone 6 mg/kg/day versus placebo (p=0.002) with a treatment difference of 0.06 [95% CI: 0.02–0.10] rises/second from baseline at 24 weeks. The study also demonstrated superiority of both vamorolone dose levels (2 and 6 mg/kg/day) versus placebo across multiple secondary endpoints. Importantly, vamorolone showed a favorable safety and tolerability profile compared to prednisone. The 24-week safety analysis indicates that treatment with vamorolone preserves growth trajectory and has no adverse impact on biomarkers of bone health compared to prednisone. Vamorolone did not stunt

growth, as validated in the current 24-week study, in which vamorolone 6 mg/kg/day versus prednisone 0.75 mg/kg/day showed a significant difference in growth velocity (p=0.02). Furthermore, statistically significant differences between vamorolone (2 and 6 mg/kg/day) and prednisone groups were seen at week 24 in biomarkers assessing bone health: osteocalcin, Procollagen 1 N-Terminal Propeptide (P1NP) and Type I Collagen C-Telopeptides (CTX) (p<0.001 for vamorolone both doses vs prednisone for all three parameters). On the basis of the available data, vamorolone could emerge as a promising alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD.

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

About VISION-DMD

VISION-DMD is a 48-week Phase 2b study designed as a pivotal trial to demonstrate efficacy and safety of vamorolone (2 and 6 mg/kg/day) versus prednisone (0.75 mg/kg/day) and placebo in 121 ambulant boys aged 4 to <7 years with Duchenne muscular dystrophy (DMD). The topline results cover data from the first period of 24 weeks of the study where vamorolone is compared to placebo (FDA-prerequisite for filing an NDA) and prednisone. The primary endpoint of the study is TTSTAND velocity at 24 weeks comparing the 6 mg/kg/day dose of vamorolone to placebo. TTSTAND velocity measures the speed at which patients are able to stand up from lying in a supine position and is a strong and recognized marker for muscle function. Secondary efficacy outcome measures include TTSTAND velocity for vamorolone at the lower dose of 2 mg/kg/day, Six-Minute Walk (6MWT) and Time to Run/Walk 10 meters (TTRW) tests at 24 weeks. The VISION-DMD study continues to completion at 48 weeks. During this second period of the study, all participants receive vamorolone. Participants from the placebo and prednisone arms are randomized to either the 2 or 6 mg/kg/day dose of vamorolone and the current vamorolone arms continue on their existing dose. The final 48-week data readout is expected in Q4-2021. In addition to efficacy, the study aims to confirm the favorable tolerability profile of vamorolone with the potential to offer an alternative to current standard of care. Although corticosteroids are part of the current care recommendations for DMD, their adverse effect profile limits their use.

About Vamorolone

Vamorolone is a first-in-class dissociative steroid which retains the anti-inflammatory activity of corticosteroids while decreasing the deleterious side effects. As such, vamorolone could emerge as a promising alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD. There is substantial unmet medical need in this patient group as high-dose corticosteroids have significant systemic side effects that diminish patient quality of life.

Vamorolone was discovered by US-based ReveraGen BioPharma, Inc. and is being developed in collaboration with Santhera, which owns worldwide rights to the drug candidate in all indications. The vamorolone development program has received funding from several international non-profit foundations and patient organizations, the US National Institutes of Health, the US Department of Defense and the European Commission's Horizon 2020 program.

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 (POL6014) 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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