Santhera and ReveraGen to Present Phase 2a/2b Efficacy and New Safety Data with Vamorolone at Forthcoming Conferences

Pratteln, Switzerland, and Rockville, MD, USA, September 23, 2021 – Santhera Pharmaceuticals (SIX: SANN) and ReveraGen BioPharma, Inc (US: private) announce presentations of the positive topline results from the pivotal VISION-DMD study and new safety analyses from long-term treatment with vamorolone at the forthcoming *World Muscle Society (WMS) 2021 Virtual Annual Conference* and *The American Society for Bone and Mineral Research (ASBMR) 2021 Annual Meeting*.

Recently, Santhera and ReveraGen announced statistically highly significant and clinically relevant data from the pivotal Phase 2b VISION-DMD study in patients with Duchenne muscular dystrophy (DMD) [1-3]. In the first 24-week phase of this trial, vamorolone demonstrated statistically significant and clinically relevant improvements versus placebo across five functional outcome measures and over a three-fold dose range from 2 to 6 mg/kg/day. The efficacy data confirm earlier results from a Phase 2a long-term extension trial, where vamorolone demonstrated similar efficacy to corticoid-treated DMD-patients after 30 months of treatment [4]. Based on clinical trial results, including long-term safety data up to 30 months, vamorolone at doses up to 6 mg/kg/day was generally well-tolerated. 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 [3-6]. This unique benefit risk profile over a wide dose range may represent a promising therapeutic approach for the chronic therapy of DMD patients, allowing for an individualized and optimized treatment regimen.

Efficacy data and recent safety analyses from studies with vamorolone will be presented as follows:

World Muscle Society (WMS) 2021 Virtual Annual Conference (September 20-24, 2021)

"Vamorolone versus placebo and prednisone in Duchenne muscular dystrophy: Results from a 24-week double-blind randomized trial"
Late-breaking poster (LBP.11/EP 524, September 23, 16:30-18:30 BST)

"Vamorolone versus corticosteroid real-world experience: Comparisons of 2-year treatment period with NorthStar UK Network and CINRG Duchenne natural history study"
Late-breaking poster (LBP.08/EP 521, September 23, 16:30-18:30 BST)

"2.5-years of vamorolone treatment in Duchenne muscular dystrophy: Results of an open label long-term extension"
Oral presentation (O.3a, September 23, 14:00-15:00 BST) and poster presentation (EP.147, September 23, 16:30-18:30 BST)

The American Society for Bone and Mineral Research (ASBMR) 2021 Annual Meeting (October 1-4, 2021)

"Vamorolone, a first-in-class dissociative steroid, is associated with improved muscle strength and favorable bone and growth plate profiles in young boys with ambulatory Duchenne Muscular Dystrophy"
Oral presentation (LB-1113, October 4, 11:30-11:45 PT)

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. 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
[3] Hoffman E. Presented at virtual PPMD Annual Conference, June 22–26, 2021.
[4] Mah JK, et al. 2021. [Manuscript in progress]
[5] Hoffman E, et al. Neurology. 2019 Sep 24; 93(13): e1312–e1323
[6] Data on File. ReveraGen Biopharma, Rockville, MD

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

For further information please contact:

Santhera
Santhera Pharmaceuticals Holding AG, Hohenrainstrasse 24, CH-4133 Pratteln
public-relations@santhera.com or
Eva Kalias, Head External Communications
Phone: +41 79 875 27 80
eva.kalias@santhera.com

ReveraGen BioPharma
Eric Hoffman, PhD, President and CEO
Phone: + 1 240-672-0295
eric.hoffman@reveragen.com

Disclaimer / Forward-looking statements

This communication does not constitute an offer or invitation to subscribe for or purchase any securities of Santhera Pharmaceuticals Holding AG. This publication may contain certain forward-looking statements concerning the Company and its business. Such statements involve certain risks, uncertainties and other factors which could cause the actual results, financial condition, performance or achievements of the Company to be materially different from those expressed or implied by such statements. Readers should therefore not place undue reliance on these statements, particularly not in connection with any contract or investment decision. The Company disclaims any obligation to update these forward-looking statements.

# # #