JAMA Neurology Publishes Positive Pivotal Clinical Trial with Vamorolone in Duchenne Muscular Dystrophy

- **Results from the VISION-DMD study showing efficacy and safety of vamorolone compared to placebo and to standard of care, prednisone, has been published in JAMA Neurology**
- **Safety analysis on bone biomarkers and growth showed no negative effects of vamorolone**
- **Regulatory filings for vamorolone with the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) are planned to be completed in Q3-2022 and Q4-2022, respectively**

Pratteln, Switzerland, and Rockville, MD, USA, September 1, 2022 –Santhera Pharmaceuticals (SIX: SANN) and ReveraGen (US: Private) announce that JAMA Neurology has published positive results of the 24-week primary efficacy and safety analysis from the VISION-DMD study evaluating vamorolone, an investigational drug for the treatment Duchenne muscular dystrophy (DMD). Vamorolone met its primary endpoint by demonstrating statistically significant and clinically relevant improvement in time to stand from floor compared to placebo, the first functional milestone to deteriorate in young children with DMD. Consistent results across multiple secondary endpoints support the results of the primary endpoint. The relative efficacy of vamorolone 6 mg/kg/day was comparable to that seen with prednisone 0.75 mg/kg/day across primary and secondary efficacy endpoints. Over the 24-week treatment period, no negative impact on biomarkers of bone health and no loss of linear growth were observed with vamorolone. Vamorolone was generally safe and well tolerated. The most commonly reported adverse events versus placebo study were cushingoid features, vomiting and vitamin D deficiency. Adverse events were generally of mild to moderate severity.

“Data from the VISION-DMD study continues to validate our ambition of developing a steroidal like treatment where we can retain the efficacy of traditional corticosteroids and reduce some of the toxicities that all too often lead to the premature discontinuation of treatment in children with DMD,” said **Eric Hoffman, PhD, Professor of Pharmaceutical Sciences, Binghamton University - SUNY, and CEO of ReveraGen**.

“Corticosteroids remain a cornerstone of treatment for many children with Duchenne muscular dystrophy and data published on the VISION-DMD study are an important advancement in the development of additional treatment options for these patients,” said **Michela Guglieri, MD, Senior Lecturer and Consultant Neurologist, Newcastle University**.

“Vamorolone has been developed as a potential first-in-class dissociative steroidal therapy,” said **Shabir Hasham, MD, Chief Medical Officer of Santhera**. “This pivotal data as well as other data presented at scientific congresses during 2022 continue to define vamorolone’s differentiated safety profile.”

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 [2-5]. This mechanism has shown the potential to ‘dissociate’ efficacy from steroid safety concerns and therefore vamorolone could emerge as an alternative to existing corticosteroids, the current standard of

care in children and adolescent subjects with DMD. Vamorolone has been granted Orphan Drug status in the U.S. and in Europe for DMD, and has received Fast Track and Rare Pediatric Disease designations by the U.S. 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] Guglieri M et al (2022). JAMA Neurol. Published online August 29, 2022. doi:10.1001/jamaneurol.2022.2480. Link.
[2] Mah JK et al (2022). JAMA Netw Open. 2022;5(1):e2144178. doi:10.1001/jamanetworkopen.2021.44178
[3] Guglieri, et al (2022) JAMA. doi:10.1001/jama.2022.4315
[4] Heier CR at al (2019). Life Science Alliance DOI: 10.26508
[5] Liu X, et al (2020). Proc Natl Acad Sci USA 117:24285-24293

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare inherited X-chromosome-linked disease, which almost exclusively affects males. DMD is characterized by inflammation which is present at birth or shortly thereafter. Inflammation leads to fibrosis of muscle and is clinically manifested by progressive muscle degeneration and weakness. Major milestones in the disease are the loss of ambulation, the loss of self-feeding, the start of assisted ventilation, and the development of cardiomyopathy. DMD reduces life expectancy to before the fourth decade due to respiratory and/or cardiac failure.

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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