Santhera and ReveraGen Announce Presentations on Long-Term Efficacy and Bone Health in DMD During Vamorolone Treatment at the *2022 World Muscle Society Congress*

Pratteln, Switzerland, and Rockville, MD, USA, October 10, 2022 – Santhera Pharmaceuticals (SIX: SANN) and ReveraGen BioPharma, Inc announce presentations of data relating to long-term efficacy, tolerability and the impact of vamorolone on bone health in patients with Duchenne muscular dystrophy (DMD) over 2.5 years of treatment [1].

The data will be shared at the 2022 World Muscle Society (WMS) Congress being held October 11-15, 2022, in Halifax, Nova Scotia, Canada, as follows:

Flash poster presentations

The spine fracture burden in boys with DMD treated with the novel dissociative steroid vamorolone versus deflazacort and prednisone – Leanne Ward, MD, October 12, 14:40-14:45 ADT, #FP.03

Results of a double-blind cross-over trial of vamorolone in Duchenne muscular dystrophy (DMD) – Eric Hoffman, PhD, October 14, 14:40-14:45 ADT, #FP.27

Posters

Vamorolone's impact on height and body mass index in patients with Duchenne muscular dystrophy (DMD) – Ward L et al, October 12, 16:00-17:30 ADT, #P.71

Result of daily regimens of prednisone, deflazacort, and vamorolone on motor function in patients with Duchenne muscular dystrophy – McDonald C et al, October 14, 14:30-16:00 ADT, #P.133

Presentations and posters will be available under this link from October 11, 2022, 16:00 ADT.

"The data being presented by our academic collaborators will foster further understanding of the potential of a dissociative steroid, such as vamorolone, in maintaining muscle strength whilst addressing important safety concerns in the treatment of DMD," said **Shabir Hasham, MD, Chief Medical Officer of Santhera**.

Santhera is hosting an on-site scientific exhibit (booth #4, located in the Ballroom Salon) where medical representatives of the Company will be present throughout the conference.

About Vamorolone

Vamorolone is an investigational drug candidate with a mode of action based on binding to the same receptor as corticosteroids but modifying 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. Link.
[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 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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