Santhera Submits Marketing Authorization Application to the UK MHRA for Vamorolone in Duchenne Muscular Dystrophy

Pratteln, Switzerland, March 2, 2023 – Santhera Pharmaceuticals (SIX: SANN) announces that it has submitted a marketing authorization application (MAA) to the UK Medicines and Healthcare products Regulatory Agency (MHRA) for vamorolone for the treatment of Duchenne muscular dystrophy (DMD).

In parallel to the MAA submission to the UK MHRA, Santhera is currently preparing an application to include vamorolone for the treatment of DMD in the *Early Access to Medicines Scheme* (EAMS) in the UK. The aim of the EAMS is to provide patients with life threatening or seriously debilitating conditions access to medicines that do not yet have a marketing authorization, when there is a clear unmet medical need.

"We are proud to have completed the third submission for vamorolone in DMD to a major regulatory agency within a few months. This underpins our determination to bring a new treatment to patients in need of effective and well-tolerated therapies," said **Shabir Hasham, MD, CMO of Santhera**. "We look forward to working closely with the MHRA during the MAA and EAMS review process with the goal of quickly providing an emerging therapy to patients in the UK."

At the core of the MAA submission are positive data from the pivotal Phase 2b VISION-DMD study which comprised a (1) 24-week period to demonstrate efficacy and safety of vamorolone (2 and 6 mg/kg/day) versus placebo and prednisone (0.75 mg/kg/day), followed by a (2) 24-week period to evaluate the maintenance of efficacy and collect additional longer-term safety and tolerability data [1]. In addition, the filing includes data from three open-label studies in which vamorolone was administered at doses between 2 and 6 mg/kg/day for a total treatment period of up to 30 months [2].

In the U.S., the Food and Drug Administration (FDA) has set October 26, 2023, as the Prescription Drug User Fee Act (PDUFA) target action date upon which approval of the new drug application (NDA) for vamorolone in DMD is expected. In the EU, a corresponding MAA has been validated and is under review by the European Medicines Agency (EMA) with an expected approval in late 2023. Subject to approvals, Santhera plans to launch vamorolone in both the U.S. and the EU in Q4-2023.

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.

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. 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 [1]. 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. 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 M et al (2022) JAMA. doi:10.1001/jama.2022.4315
[4] Heier CR et 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. Corticosteroids are the current standard of care for the treatment of DMD.

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. The Company 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 Duchenne muscular dystrophy (DMD) as an alternative to standard corticosteroids. For vamorolone in the treatment of DMD, Santhera has a new drug application (NDA) under review by the U.S. FDA, a marketing authorization application (MAA) under review by the European Medicines Agency (EMA) and an MAA submitted to the UK Medicines and Healthcare products Regulatory Agency (MHRA). 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

For further information please contact:

Santhera
Santhera Pharmaceuticals Holding AG, Hohenrainstrasse 24, CH-4133 Pratteln
public-relations@santhera.com or
Eva Kalias, Head Investor Relations & 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

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