

Santhera Submits Marketing Authorization Application to the European Medicines Agency for Vamorolone in Duchenne Muscular Dystrophy

Pratteln, Switzerland, October 3, 2022 – Santhera Pharmaceuticals (SIX: SANN) announces that the Company has submitted a marketing authorization application (MAA) to the European Medicines Agency (EMA) for vamorolone for the treatment of Duchenne muscular dystrophy (DMD).

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 prednisone (0.75 mg/kg/day) and placebo, 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].

“Duchenne is a debilitating disease with patients in need for effective and well-tolerated therapies,” said **Shabir Hasham, MD, CMO of Santhera**. “Our EU submission marks an important step towards making vamorolone available to DMD patients in the respective European markets and we look forward to working closely with the EMA during the review process.”

In the U.S., Santhera expects to complete the filing of the new drug application (NDA) for vamorolone in DMD to the Food and Drug Administration (FDA) in Q4-2022.

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.

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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