European Medicines Agency Validates Santhera's Marketing Authorization Application for Vamorolone in Duchenne Muscular Dystrophy

Pratteln, Switzerland, October 31, 2022 – Santhera Pharmaceuticals (SIX: SANN) announces that the European Medicines Agency (EMA) has validated its marketing authorization application (MAA) for vamorolone for the treatment of Duchenne muscular dystrophy (DMD). Validation confirms that the submission is complete and that the review by the EMA's Committee for Medicinal Products for Human Use (CHMP) has begun.

"The initiation of the CHMP review of our application is an important milestone for patients with DMD and a significant acknowledgement for our Company. We are looking forward to working closely with the rapporteurs and CHMP during the review process to make vamorolone available to patients as soon as possible," said **Shabir Hasham, MD, CMO of Santhera**.

Santhera expects the CHMP to complete the review and issue an opinion regarding approval to EMA's European Commission (EC) in late Q3-2023. Subject to EC approval, expected later in 2023, vamorolone will receive marketing authorization in all member states of the European Union, as well as in Norway, Liechtenstein and Iceland.

In the U.S., Santhera and ReveraGen have just completed the submission of the new drug application (NDA) to the Food and Drug Administration (FDA), seeking priority review for vamorolone for the treatment of DMD. Subject to approval, this paves the way for a potential launch in H2-2023.

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 [1-5]. 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 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 as an alternative to standard corticosteroids. Santhera has submitted a new drug application (NDA) to the U.S. FDA and a marketing authorization application (MAA) to the European Medicines Agency (EMA) for vamorolone for the treatment of DMD. 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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