# Vamorolone NDA mid-cycle review meeting by FDA completed

**Pratteln, Switzerland, and Rockville, MD, USA, April 25, 2023 – Santhera Pharmaceuticals (SIX: SANN) and ReveraGen BioPharma, Inc announce the successful completion of the mid-cycle review meeting by the U.S. Food and Drug Administration (FDA) of the new drug application (NDA) for vamorolone for the treatment of Duchenne muscular dystrophy (DMD).**

At the recent mid-cycle review meeting, the FDA indicated that no significant review or safety concerns were noted up to that point in their ongoing review. The FDA re-affirmed its earlier decision to forgo an Advisory Committee Meeting. In addition, the FDA confirmed the PDUFA date of October 26, 2023, on which an approval decision on the vamorolone NDA is expected.

As part of the ongoing NDA review, the FDA conducted several inspections at various sites, including the contract manufacturer, the sponsor and certain clinical trial sites. All inspections to date were concluded with satisfactory outcomes.

“We are very pleased to have completed this stage of the review process,” said **Shabir Hasham, MD, Chief Medical Officer of Santhera**. “We look forward to working closely with the FDA during the remainder of the review process and are confident about being able to provide an emerging therapy to patients with DMD in the U.S. soon.”

“The progress we have made and the positive interactions with the FDA to date give us a real sense of accomplishment,” said **Eric Hoffman, PhD, President and CEO of ReveraGen BioPharma**. “Step by step we have come even closer to bringing a novel therapy to patients that, if approved, would contribute to improving the current standard of care in DMD.”

In Europe, the review of the marketing authorization application (MAA) for vamorolone by the European Medicines Agency (EMA) is on track. A CHMP opinion is expected in Q4-2023, followed by an approval decision by the European Commission (EC) in late 2023. In the UK, a corresponding MAA is under review by the Medicines and Healthcare products Regulatory Agency (MHRA). Subject to approvals, Santhera plans to launch vamorolone in both the U.S. and the EU in late 2023.

Vamorolone has been granted Orphan Drug status for DMD in the U.S. and in Europe 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 glucocorticoids 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. 2022;79(10):1005-1014. 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 (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

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