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Ad hoc announcement pursuant to Art. 53 LR

Santhera Updates on Ongoing NDA Submission for Vamorolone

Pratteln, Switzerland, June 29, 2022 – Santhera Pharmaceuticals (SIX: SANN) announces that the clinical module of its rolling new drug application (NDA) for vamorolone in Duchenne muscular dystrophy (DMD) has been submitted to the U.S. Food and Drug Administration (FDA). Finalization of the NDA submission to start the FDA official review is postponed by 4-6 months to Q4-2022 after a third-party contract manufacturing organization (CMO) communicated a delay in establishing FDA-inspection readiness.

Santhera commenced the NDA filing as a rolling submission in March 2022 following a successful pre-NDA meeting with the FDA. In its conclusions from this meeting, the FDA considered the proposed clinical efficacy and safety data sufficient to support an NDA filing of vamorolone for the treatment of DMD. The rolling submission has been proceeding according to plan with the clinical module submitted on June 28, 2022.

A third-party contract manufacturing organization has informed Santhera that establishing preparedness for an FDA pre-approval inspection is delayed until later this year. Formal confirmation of inspection readiness by the CMO is a prerequisite for the FDA to consider a filing complete and to accept an NDA for review. Therefore, Santhera will discuss with the Agency the status and updated timelines for completing the NDA filing by Q4-2022.

In Europe, preparations for a marketing authorization application (MAA) for vamorolone for the treatment of DMD to the European Medicines Agency (EMA) are proceeding according to plan.

Vamorolone has been granted Orphan Drug status in the US and in Europe for DMD, and has received Fast Track and Rare Pediatric Disease designations by the US 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.

About Vamorolone

Vamorolone is a drug candidate with a mode of action that binds to the same receptor as corticosteroids but modifies its downstream activity and as such is considered a dissociative anti-inflammatory drug [1-4]. This mechanism has the potential to 'dissociate' efficacy from typical steroid safety concerns and therefore vamorolone could emerge as a promising alternative to existing corticosteroids, the current standard of care in children and adolescents 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. 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.

References:
[1] Mah JK et al (2022). JAMA Netw Open. 2022;5(1):e2144178. doi:10.1001/jamanetworkopen.2021.44178
[2] Guglieri, et al (2022) JAMA. doi:10.1001/jama.2022.4315
[3] Heier CR at al (2019). Life Science Alliance DOI: 10.26508
[4] 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. 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.

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