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# Santhera Announces Acceptance by Swissmedic of Marketing Authorization Application for AGAMREE® (vamorolone) in Duchenne Muscular Dystrophy

**Pratteln, Switzerland, September 24, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces that Swissmedic, the Swiss Agency for Therapeutic Products, has accepted for review the marketing authorization application (MAA) for AGAMREE® (vamorolone) for the treatment of Duchenne muscular dystrophy (DMD).**

Santhera asked Swissmedic to assess AGAMREE® as part of Article 13 TPA (therapeutics product act) procedure, allowing it to consider the results of foreign regulatory authorities' assessments when reviewing a medicinal product for authorization in Switzerland. In the case of AGAMREE, Swissmedic will consider the EU approval of the drug received in December 2023. This can streamline the approval process by leveraging existing evaluations from countries with comparable regulatory standards. The outcome of the Swissmedic review is expected in late H1-2026 with the possibility to be accelerated to early 2026 if the Article 13 is accepted. Swissmedic approval in addition to EMA/FDA/MHRA approvals will leverage the access for this orphan drug to other markets through an abbreviated review.

"Submission of AGAMREE for DMD to Swissmedic is an important milestone, following approvals from the U.S. FDA, the European Commission in the EU, and the UK MHRA, in addition to acceptance for priority review from China's NMPA," commented **Shabir Hasham, MD, Chief Medical Officer of Santhera.** "We look forward to working closely with Swissmedic to address the high unmet need for improved treatment options for patients suffering from DMD in Switzerland."

**About AGAMREE® (vamorolone)**

AGAMREE is a novel drug with a mode of action based on binding to the same receptor as glucocorticoids but modifying its downstream activity. Moreover, it is not a substrate for the 11-β-hydroxysteroid dehydrogenase (11β-HSD) enzymes that may be responsible for local drug amplification and corticosteroid-associated toxicity in local tissues [1-4]. This mechanism has shown the potential to 'dissociate' efficacy from steroid safety concerns and therefore AGAMREE is positioned as a dissociative anti-inflammatory drug and an alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD [1-4].

In the pivotal VISION-DMD study, AGAMREE 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, 4]. The most commonly reported side effects were cushingoid features, vomiting, weight increase and irritability. Side effects were generally of mild to moderate severity.

Currently available data show that AGAMREE, unlike corticosteroids, has no restriction of growth [5] and no negative effects on bone metabolism as demonstrated by normal bone formation and bone resorption serum markers [6].

AGAMREE (vamorolone), an orphan medicinal product, is approved for use in the United States (Prescribing Information), the European Union (Summary of Product Characteristics) and the United Kingdom.

References:
[1] Dang UJ et al. (2024) Neurology 2024;102:e208112. doi.org/10.1212/WNL.0000000000208112. Link.
[2] Guglieri M et al (2022). JAMA Neurol. 2022;79(10):1005-1014. doi:10.1001/jamaneurol.2022.2480. Link.
[3] Liu X et al (2020). Proc Natl Acad Sci USA 117:24285-24293
[4] Heier CR et al (2019). Life Science Alliance DOI: 10.26508
[5] Ward et al., WMS 2022, FP.27 - Poster 71. Link.
[6] Hasham et al., MDA 2022 Poster presentation. Link.

**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 diseases with high unmet medical need. The Company has an exclusive license from ReveraGen for all indications worldwide to AGAMREE® (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. AGAMREE for the treatment of DMD is approved in the U.S. by the Food and Drug Administration (FDA), in the EU by the European Medicines Agency (EMA), and in the UK by the Medicines and Healthcare products Regulatory Agency (MHRA). Santhera has out-licensed rights to AGAMREE for North America to Catalyst Pharmaceuticals and for China to Sperogenix Therapeutics. For further information, please visit www.santhera.com.

*AGAMREE® is a trademark of Santhera Pharmaceuticals.*

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