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Santhera's AGAMREE® (Vamorolone) Approved in the European Union for the Treatment of Duchenne Muscular Dystrophy

- *European Commission (EC) grants marketing authorization for AGAMREE® for the treatment of Duchenne muscular dystrophy (DMD) in patients aged 4 years and older*
- *AGAMREE® is the only approved medication in the European Union (EU) for treating DMD, and the first DMD treatment approved in both the U.S. and EU*
- *First commercial launch of AGAMREE®, in Germany, expected in Q1-2024*
- *EMA acknowledges safety benefits of AGAMREE® with regards to preserving bone health and maintaining growth compared to standard of care corticosteroids*

Pratteln, Switzerland, December 18, 2023 – Santhera Pharmaceuticals (SIX: SANN) announces that AGAMREE® (vamorolone) has been approved in the European Union (EU) for the treatment of Duchenne muscular dystrophy (DMD) in patients 4 years of age and older, independent of the underlying mutation and ambulatory status. The European Medicines Agency (EMA) acknowledged clinically important safety benefits of AGAMREE with regards to maintaining normal bone metabolism, density and growth compared to standard of care corticosteroids, alongside similar efficacy [1]. In addition, patients who switched from a standard of care corticosteroid to AGAMREE maintained the efficacy benefit while recovering their growth and bone health.

This makes AGAMREE® the first and only medicinal product to have received full approval in the EU and, following U.S. Food and Drug Administration (FDA) approval in October, the first authorized treatment in both the U.S. and EU to treat patients with DMD.

"We are thrilled to receive approval from the EC to bring AGAMREE to patients in the EU, which highlights the favorable safety and tolerability profile over conventional corticosteroids, including benefits for bone health and growth," said **Dario Eklund, CEO of Santhera**. "Our team is now focused on ensuring AGAMREE is made available to the Duchenne patients as soon as possible, with a first commercial launch planned for Germany in Q1-2024."

"World Duchenne Organization is happy to see the development of AGAMREE, during which the involvement of many Duchenne patient organizations has been instrumental by providing early funding and contributing to clinical trial design, outcomes and recruitment, led to the first full market authorization in the European Union for treating Duchenne muscular dystrophy," said **Elizabeth Vroom, Chair of the World Duchenne Organization**.

The approval by the EC was based on data from the positive pivotal VISION-DMD study and 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. In the pivotal VISION-DMD study, boys treated with vamorolone on average maintained growth similar to those treated with placebo, whilst those treated with prednisone on average experienced growth stunting. Patients who switched from prednisone to vamorolone after 24-weeks were, on average, able to resume growing in height over the remainder of the study.

Unlike corticosteroids, vamorolone did not result in a reduction of bone metabolism as measured by bone biomarkers, nor in a significant reduction of bone mineralization in the spine as measured by Dual Energy X-Ray Absorptiometry (DXA) after 48 weeks in the clinical studies.

Santhera will continue to collect data to further characterize the long-term effectiveness and the broader safety differentiation of vamorolone.

The EC's decision follows the positive opinion for AGAMREE from the Committee for Medicinal Products for Human Use (CHMP) which was announced on October 13, 2023. The marketing authorization will be valid in all 27 member states of the EU as well as Iceland, Liechtenstein and Norway, with Santhera expecting to begin a commercial launch in Germany in Q1-2024.

Santhera announced on October 27, 2023 that the FDA approved AGAMREE oral suspension 40 mg/ml in the United States for the treatment of DMD in patients 2 years of age and older. Catalyst Pharmaceuticals holds an exclusive license for AGAMREE in North America and plans to launch the product in the U.S. in Q1-2024.

About AGAMREE® (vamorolone)

Vamorolone is a novel drug with a mode of action based on binding to the same receptor as glucocorticoids but modifying its downstream activity and is not a substrate for the 11-β-hydroxysteroid dehydrogenase (11β-HSD) enzymes that may be responsible for local tissue amplification and corticosteroid-associated toxicity in local tissues [2-4]. This mechanism has shown the potential to 'dissociate' efficacy from steroid safety concerns and therefore vamorolone is positioned as an alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD [2-4].

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 [3]. 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.

Currently available data show that vamorolone, 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) has 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 MHA for DMD. Vamorolone is approved for use in the United States and the European Union.

References:

[1] European public assessment report (EPAR), summary of product characteristics (SmPC)
[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 and pulmonary 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) and in the EU by the European Medicines Agency (EMA); the marketing authorization application (MAA) is under review by the UK Medicines and Healthcare products Regulatory Agency (MHRA). Santhera has out-licensed rights to vamorolone for North America to Catalyst Pharmaceuticals and for China to Sperogenix Therapeutics. The clinical stage pipeline also includes lonodelestat to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases. For further information, please visit www.santhera.com.

AGAMREE® is a trademark of Santhera Pharmaceuticals.

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