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Santhera Receives Approval for AGAMREE® (Vamorolone) as a Treatment for Duchenne Muscular Dystrophy in the United Kingdom

- *Approval by the Medicines and Healthcare products Regulatory Agency (MHRA) of AGAMREE® in the United Kingdom follows marketing authorization for this medicine in the EU and U.S.*
- *MHRA acknowledges safety benefits of AGAMREE with regards to preserving bone health and maintaining growth compared to standard of care corticosteroids*
- *Launches of AGAMREE in Europe, with Germany taking the lead, are planned to start in Q1-2024*

Pratteln, Switzerland, January 12, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces that AGAMREE® (vamorolone) has been approved in the United Kingdom (UK) 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 UK's MHRA, adopting the view of 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].

"We are delighted to have secured a third approval for AGAMREE to treat Duchenne from a major regulatory agency, after the U.S. FDA and the EU EMA, within a couple months," said **Shabir Hasham, MD, Chief Medical Officer of Santhera**. "In addition to its anti-inflammatory efficacy, both the EMA and the MHRA recognize the benefits of treatment with AGAMREE for bone health and growth, underlining the favorable safety and tolerability profile of this novel medicine compared to conventional corticosteroids. We are working towards making AGAMREE available to patients in the UK in the second half-year 2024, after NICE completes its pricing review. Initial European launch will be in Germany in Q1."

"We are delighted that the first drug designed specifically for everyone with Duchenne has been approved in the UK," said **Emily Reuben OBE, Chief Executive of Duchenne UK**, and **Alex Johnson OBE, Chief Executive of Joining Jack**, who are the co-founders of Duchenne UK. "When our sons were diagnosed with Duchenne, we were told that steroids, the standard medication for children with it, would keep them independently mobile for longer. But with harmful side effects. We didn't think this was good enough, and invested in finding better treatments. Duchenne UK and our partner charities, Joining Jack and the Duchenne Research Fund, funded the early-stage clinical research to test vamorolone in patients, when no-one else would. For it now to be available in the UK to treat DMD is proof that we can find better treatments for Duchenne and change things for Duchenne."

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. In addition, patients who switched from a standard of care corticosteroid to AGAMREE maintained the efficacy benefit while recovering their growth and bone health.

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

This approval follows the approval of AGAMREE by the U.S. Food and Drug Administration (FDA) for the treatment of DMD in patients aged 2 years and older in the U.S. and approval by the European Commission for the treatment of DMD in the EU in patients aged 4 years and older. This makes AGAMREE the first and only medicinal product fully approved in the EU and UK for DMD, and the first treatment approved for the treatment of DMD all three geographies.

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, Fast Track and Rare Pediatric Disease designations by the U.S. FDA and Promising Innovative Medicine (PIM) status from the UK MHRA for DMD. AGAMREE is approved for use in the United States, the European Union and the United Kingdom.

References:

[1] Summary of Product Characteristics (SmPC) / European public assessment report (EPAR)
[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), in the EU by the European Medicines Agency (EMA) and in the United Kingdom 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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