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Santhera Launches AGAMREE® (Vamorolone) in Germany as First Market for the Treatment of Duchenne Muscular Dystrophy

- *AGAMREE® is the only approved medication in the European Union (EU) for treating all patients from age 4 years with DMD, and the first DMD treatment approved across the U.S., EU and UK*
- *This marks the first commercial launch of AGAMREE® globally*

Pratteln, Switzerland, January 15, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces the launch of AGAMREE® (vamorolone) for the treatment of Duchenne muscular dystrophy (DMD) in patients 4 years of age and older, irrespective of the underlying mutation and ambulatory status, in Germany.

AGAMREE® is the first and only medicinal product for DMD to have received full approval in the EU and, following approval in the U.S. last October and in the UK last week, it is the first authorized treatment for patients with the disease in all three territories.

"We are very happy that AGAMREE is now commercially available in Germany, the first country worldwide, for the treatment of DMD," **said Dario Eklund, CEO of Santhera**. "This significant milestone represents Santhera's commitment to fill the unmet need in DMD and provide a safe and effective treatment for DMD patients. For Santhera, this launch signifies a leap forward as the Company enters the commercial stage. In parallel, we continue to work towards our own staged commercial roll-out in the large European markets, alongside ongoing discussions with distribution partners for other regions."

Silvia Hornkamp, Managing Director of the German Duchenne Foundation, commented: "We are delighted that Santhera has been investing in Duchenne research for so many years. Now the time has come—with the new drug AGAMREE, patients suffering from Duchenne muscular dystrophy have access to a better tolerated alternative to steroids. It is a benefit for the sick children, who until now have been burdened with many side effects due to the long-term use of steroids."

This launch follows the European Commission's approval of AGAMREE® on December 18, 2023, for all 27 EU member states as well as Iceland, Liechtenstein, and Norway. As part of the marketing authorization, 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. Patients treated with AGAMREE or placebo showed normal and similar growth while growth stunting was observed in children treated with prednisone. In addition, patients who switched from a standard of care corticosteroid to AGAMREE maintained the efficacy benefit while recovering their growth and bone health.

For more information about AGAMREE in the European Union, please see the Summary of Product Characteristics (SmPC) [1].

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, the European Union and the United Kingdom.

References:
[1] Summary of Product Characteristics (SmPC). English. German.
[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 UK by the 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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