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Santhera's Partner Catalyst Pharmaceuticals Launches AGAMREE® (Vamorolone) in the United States

- *AGAMREE® is U.S. FDA approved and now available in the United States for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older*
- *This follows the first commercial launch of AGAMREE in Germany in January 2024 by Santhera*
- *AGAMREE is the first DMD treatment approved across the U.S., EU and UK*

Pratteln, Switzerland, March 14, 2024 – Santhera Pharmaceuticals (SIX: SANN) is pleased to note the launch of AGAMREE® (vamorolone) for the treatment of Duchenne muscular dystrophy (DMD) in the United States (U.S.) by Catalyst Pharmaceuticals, Inc. (NASDAQ: CPRX), the Company's commercialization partner for North America.

"Congratulations to our partner Catalyst on the launch of AGAMREE® in the United States. Following the first global market introduction of this novel product in Germany in January, this launch represents an important next step in making AGAMREE available to as many patients with DMD as soon as possible," said **Dario Eklund, CEO of Santhera**.

The launch of AGAMREE in the U.S. follows the approval of the product by the U.S. Food and Drug Administration (FDA) on October 26, 2023, based on data from the pivotal VISION-DMD study, recently published in *Neurology*, and supplemented with safety information collected from three open-label studies, including extension studies.

According to the license agreement between the companies, first announced in June 2023, Catalyst holds an exclusive North American license to commercialize AGAMREE for DMD and all potential future indications. Under the agreement, Catalyst will pay Santhera sales-based milestones of up to USD 105 million as well as up to low-teen percentage royalties and will assume Santhera's corresponding third-party royalty obligations on AGAMREE sales in all indications in North America.

Read more in Catalyst's announcement here.

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 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, Inc. 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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