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Santhera Announces Approval from China's NMPA for AGAMREE® (Vamorolone) as a Treatment for Duchenne Muscular Dystrophy

- *China's National Medical Products Administration (NMPA) approved AGAMREE® for the treatment of Duchenne muscular dystrophy in patients aged 4 years and older*
- *This approval makes AGAMREE the first and only approved DMD therapy in China*

Pratteln, Switzerland, December 11, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces that China's National Medical Products Administration (NMPA) has approved AGAMREE® (vamorolone) for use in China in patients aged 4 years and older.

"DMD is a devastating condition affecting over 70,000 families in China, and until now, there had been no approved treatment option for patients," said **Dario Eklund, CEO of Santhera**. "We are delighted that NMPA has approved AGAMREE for the treatment of DMD in China and we look forward to working with our partner Sperogenix Therapeutics as it prepares for the commercialization of the product to ensure DMD patients in China can benefit as soon as possible."

Mr. Yan Zhiyu, Co-founder, Chairman and CEO of Sperogenix Therapeutics, stated: "The approval of AGAMREE through the Priority Review Program reflects the government's high level of attention to the development of rare disease drugs, and also reflects Sperogenix's firm commitment to the rare disease patients in China who are in urgent unmet needs. The approval of AGAMREE is an important milestone, and we will continue to uphold our commitment to patient needs and work with stakeholders to accelerate supply and access initiatives, so that more DMD patient families can benefit as soon as possible."

AGAMREE is the first and only approved therapy for the treatment of DMD in China. This approval follows the acceptance of the new drug application (NDA) filing for AGAMREE in DMD for patients aged 4 years and older by the National Medical Products Administration (NMPA) in March 2024, acknowledging clinically important safety benefits with regards to maintaining normal bone metabolism, density, and growth compared to standard of care corticosteroids. AGAMREE has also been incorporated into both the Priority Review Program and the Breakthrough Therapy Program, which addresses serious diseases lacking effective treatments and includes drugs offering clear clinical advantages over existing treatments.

According to the license agreement between the companies first announced in January 2022, Sperogenix holds exclusive development and commercialization rights to AGAMREE in DMD and all other rare disease indications for China. Santhera is supplying treatment medication to Sperogenix for the EAP as well as for commercialization. Sperogenix will pay Santhera double-digit percentage royalties on net product sales (including for the EAP) and additional revenue-dependent milestones on commercial sales.

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].

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.

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About Sperogenix Therapeutics

Founded in 2019, Sperogenix Therapeutics is a platform company dedicated to developing and commercializing genetic disease and rare disease therapeutics in China. With prioritized therapeutic areas such as neuromuscular diseases and inherited metabolic diseases, Sperogenix is dedicated to establishing an innovative commercial model tailored to the China rare disease field, in order to provide affordable and reliable products and services to Chinese physicians and patients. In 2022, Sperogenix Therapeutics obtained the exclusive development and commercialization rights from Santhera Pharmaceuticals for AGAMREE® in DMD and all other rare diseases in the Greater China region (including Hong Kong, Macao and Taiwan) and the Southeast Asian region and production rights in all of the above regions under certain conditions. www.sperogenix.com.

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