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Santhera Announces Approval in Hong Kong for AGAMREE® (Vamorolone) as a Treatment for Duchenne Muscular Dystrophy

- *Department of Health (DoH) of Hong Kong approved AGAMREE® for the treatment of Duchenne muscular dystrophy (DMD) in patients aged 4 years and older*
- *Follows recent approval from China's National Medical Products Administration (NMPA)*

Pratteln, Switzerland, December 20, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces that the Department of Health of Hong Kong, China, has approved AGAMREE® (vamorolone) for use in patients aged 4 years and older.

The approval by the DoH of Hong Kong constitutes the fifth independent approval by local health authorities after the US FDA, EMEA, MHRA and the NMPA and will allow patients with DMD in Hong Kong access to AGAMREE in the near future.

According to the license agreement between the companies first announced in January 2022, Sperogenix holds exclusive rights for the development and commercialization of AGAMREE for DMD and other rare disease indications in Greater China. This agreement was amended in March 2023 to include the option for several additional Southeast Asian countries, namely Brunei, Cambodia, Timor-Leste (East Timor), Indonesia, Laos, Malaysia, Myanmar (Burma), the Philippines, Singapore, Thailand, and Vietnam in exchange for a USD 4 million payment by Sperogenix. Sperogenix exercised this option in July 2024 to include these territories in their license agreement. Santhera is supplying AGAMREE to Sperogenix for both the ongoing Early Access Program (EAP) and for commercialization. Sperogenix will pay Santhera royalties at a double-digit percentage on net product sales, along with additional revenue-dependent milestones based 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), in the UK by the Medicines and Healthcare products Regulatory Agency (MHRA), in China by the National Medical Products Administration (NMPA) and Hong Kong by the DoH. Santhera has out-licensed rights to AGAMREE for North America to Catalyst Pharmaceuticals and for China and certain countries in Southeast Asia to Sperogenix Therapeutics. For further information, please visit www.santhera.com.

AGAMREE® is a trademark of Santhera Pharmaceuticals.

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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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