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Santhera Announces Launch of Early Access Program in China for AGAMREE® by its Partner Sperogenix

Pratteln, Switzerland, June 10, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces that its partner Sperogenix Therapeutics has launched a paid-for Early Access Program (EAP) for AGAMREE® (vamorolone) in China for patients with Duchenne muscular dystrophy (DMD).

In April 2024, the Hainan Medical Products Administration (HMPA) authorized the EAP for AGAMREE based on local policies, AGAMREE's existing overseas approvals (U.S., EU, UK) and its demonstrated ability to address urgent clinical needs in DMD, where approved treatments are currently unavailable in China. The EAP has started in the Bo'ao Lecheng Pilot Zone, located in Hainan Province, in mid-May, when the first patients were treated with AGAMREE.

In March 2024, the National Medical Products Administration (NMPA) accepted the NDA filing for AGAMREE in DMD for patients aged 4 years and older, incorporating it into both the Priority Review Program and the Breakthrough Therapy Program. Subject to a positive review outcome, approval could be obtained by Q1-2025.

Duchenne muscular dystrophy is a rare neuromuscular disease affecting about 70,000 patients in China. Currently, there is no approved drug to treat DMD in China, leaving a high unmet medical need and therapeutic gap, especially considering the increasing diagnosis rates that enable more patients to access specialized treatment centers.

According to the license agreement between the companies, first announced in January 2022, Sperogenix holds exclusive development and commercialization rights to vamorolone 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].

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 Sperogenix

Sperogenix Therapeutics is a platform company dedicated to developing and commercializing 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. Sperogenix was founded in 2019 and is backed by biopharma industry blue chip investors including Lilly Asia Ventures, Morningside Ventures and Prosperico Ventures. www.sperogenix.com.

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 AGAMREE for North America to Catalyst Pharmaceuticals, Inc. and for China to Sperogenix Therapeutics. For further information, please visit www.santhera.com.

AGAMREE® is a trademark of Santhera Pharmaceuticals.

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