Santhera Pharmaceuticals Holding AG
Hohenrainstrasse 24, 4133 Pratteln, Switzerland

Phone: +41 61 906 89 50 | Fax: +41 61 906 89 51
www.santhera.com

Santhera Announces NDA for Vamorolone in Duchenne Muscular Dystrophy Accepted and Granted Priority Review by China's NMPA

Pratteln, Switzerland, March 27, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces that the China National Medical Products Administration (NMPA) has accepted for priority review the new drug application (NDA) for vamorolone in Duchenne muscular dystrophy (DMD) which was submitted by Sperogenix Therapeutics, Santhera's specialized rare disease partner for China.

The Center for Drug Evaluation (CDE) of the Chinese drug authority NMPA accepted the filing and granted priority review for vamorolone in DMD for patients aged 4 years and older which could, subject to a positive outcome, lead to approval by Q1 2025. Previously, the CDE included vamorolone for the treatment of DMD in the Breakthrough Therapy Program, which addresses serious diseases lacking effective treatments and includes drugs offering clear clinical advantages over existing treatments.

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.

The NDA in China is supported by an extensive data package which led to approval of vamorolone in the U.S., EU and UK in late 2023/early 2024. Evidence for clinical efficacy and safety was based on data from the positive pivotal VISION-DMD study, showing superiority of vamorolone compared with placebo, and three open-label studies in which vamorolone was administered at doses between 2 and 6 mg/kg/day for a total treatment period of up to 30 months. In addition, the filing included data from three open-label studies in which vamorolone was administered at doses between 2 and 6 mg/kg/day for a total treatment period of up to 30 months [1-4]. The submission is further supported by a study which investigated the pharmacokinetic parameters of vamorolone in healthy adult Chinese volunteers.

According to the license agreement between the companies, first announced in January 2022, Sperogenix Therapeutics holds exclusive development and commercialization rights to vamorolone in DMD and all other rare disease indications for China. Upon commercialization, Sperogenix will pay Santhera sales-based milestones and double-digit percentage royalties on net 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 Properico Ventures.

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. 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.

For further information please contact:
public-relations@santhera.com or
Eva Kalias, Head Investor Relations & Communications
Phone: +41 79 875 27 80
eva.kalias@santhera.com

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