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Santhera shares updates on commercial rollout of AGAMREE®

Pratteln, Switzerland, April 16, 2025 – Santhera Pharmaceuticals (SIX: SANN) announces the latest progress on the commercial rollout of AGAMREE® for the treatment of Duchenne Muscular Dystrophy (DMD).

Funding from National Health Service (NHS) England is now available for AGAMREE for the treatment of DMD, enabling prescribing in England to commence. This follows positive NICE (National Institute for Health and Care Excellence) final guidance for AGAMREE announced on January 16, 2025 and initial sales already achieved in Scotland and Wales.

In addition, Santhera notes that Kye Pharmaceuticals, sub-licensee of its commercialization partner Catalyst Pharmaceuticals, has received acceptance by Health Canada for the New Drug Submission for AGAMREE with Priority Review. This potentially paves the way for the therapy to become the first approved treatment for DMD in Canada by the end of 2025.

Dario Eklund, CEO of Santhera said: "I'm pleased to share the latest progress on the commercial rollout of AGAMREE, particularly in the UK, where a significant portion of our clinical trials were conducted. These trial sites played a key role in generating the data to support the approval. We are proud to see this milestone translate into access for patients in England living with DMD."

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].

▼ *This medicinal product is subject to additional monitoring. This will allow quick identification of new safety information. Healthcare professionals are asked to report any suspected adverse reactions.*

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 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 Department of Health (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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