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Santhera Receives Positive NICE Final Guidance for AGAMREE® (Vamorolone) as a Treatment for Duchenne Muscular Dystrophy

- *Santhera has begun launch preparations for AGAMREE® in UK with first sales expected in this quarter*

Pratteln, Switzerland, January 16, 2025 – Santhera Pharmaceuticals (SIX: SANN) announces that the National Institute for Health and Care Excellence (NICE) has issued positive Final Guidance that recommends AGAMREE® (vamorolone) for use in the National Health Service (NHS) in England, Wales and Northern Ireland for the treatment of Duchenne muscular dystrophy (DMD) in patients 4 years of age and older.

This follows confirmation that no appeals were received against the Final Draft Guidance (FDG) recommendation announced on December 10, 2024. Following this, Santhera has already started launch preparations for AGAMREE in the UK and expect first sales in this quarter.

AGAMREE is the first and only medicinal product for DMD to have received full approval in the EU, US, and UK. This recommendation follows the Medicines and Healthcare products Regulatory Agency's (MHRA) approval of AGAMREE on January 11, 2024, which, alongside the European Medicines Agency (EMA), acknowledged clinically important tolerability benefits with regards to maintaining normal bone metabolism, density, and growth compared to standard of care corticosteroids, alongside similar efficacy from clinical trials.

The Final Guidance is available on the NICE website: www.nice.org.uk/guidance/TA1031

For more information about AGAMREE in Great Britain, including Scotland: Summary of Product Characteristics

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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