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

- *AGAMREE® has been recommended by NICE for treating Duchenne muscular dystrophy (DMD) in patients 4 years of age and older in England, Wales and Northern Ireland*
- *Santhera will be working closely with NHS England, NHS Wales and NHS Northern Ireland to ensure rapid patient access*
- *Santhera also pursuing reimbursement via the Scottish Medicines Consortium (SMC) to ensure access for patients in Scotland*

Pratteln, Switzerland, December 10, 2024 – Santhera Pharmaceuticals (SIX: SANN) announces that the National Institute for Health and Care Excellence (NICE) has issued 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.

"DMD is a devastating condition, the most common and severe form of muscular dystrophy, and patients urgently need improved treatment options," said **Dario Eklund, CEO of Santhera**. "We are delighted that NICE has recommended AGAMREE for patients with DMD, and we are committed to working closely with the NHS to ensure patients can access AGAMREE as quickly as possible."

Emily Reuben, Chief Executive of Duchenne UK, stated: "We are very proud that a treatment that we have supported for so long will now be available in the NHS. The approval of vamorolone (AGAMREE) is the culmination of a global effort of scientists, clinicians and patient advocacy groups investing in and supporting the development of vamorolone."

Professor Michela Guglieri, Consultant Neurologist at Newcastle Hospitals NHS Foundation Trust, added: "AGAMREE represents a significant advancement for the treatment of DMD, offering patients an alternative option to slow down the progression of the disease while reducing some of the side effects of traditional corticosteroids that affect patient's quality of life. This recommendation by NICE is a crucial step forward in improving care for DMD patients across England, Wales and Northern Ireland."

With this positive recommendation, AGAMREE is expected to be funded and available for use within 90 days in England, Wales and Northern Ireland. The Company is also progressing through the reimbursement process with the Scottish Medicines Consortium (SMC) to secure access to AGAMREE for patients in Scotland.

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 safety benefits with regards to maintaining normal bone metabolism, density, and growth compared to standard of care corticosteroids, alongside similar efficacy from clinical trials.

For more information about AGAMREE:
For Great Britain: Summary of Product Characteristics
For Northern Ireland and European Union: 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 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), 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 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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