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Santhera Receives Positive CHMP Opinion to Expand AGAMREE® (vamorolone) Use in Pediatric DMD Patients Aged Two and Older in the EU

Pratteln, Switzerland, April 27, 2026 – Santhera Pharmaceuticals (SIX: SANN) today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending an extension to the marketing authorization for AGAMREE® (vamorolone) to include the treatment of patients with Duchenne muscular dystrophy (DMD) from 2 years of age.

AGAMREE is currently approved in the European Union for the treatment of DMD in patients 4 years of age and older. If confirmed by the European Commission, the CHMP opinion would expand the authorized age range to include children from 2 years of age, a population in which early anti-inflammatory intervention may offer long-term benefit, and where the tolerability burden of conventional corticosteroids remains a key concern.

Dario Eklund, Chief Executive Officer of Santhera, said: "*This positive CHMP opinion marks an important step towards our goal of making AGAMREE available to every DMD patient who could benefit. For families and clinicians, deciding when to initiate treatment in very young children can be particularly challenging, given the limited treatment options available.*"

Shabir Hasham, MD, Chief Medical Officer of Santhera, said: "*Initiating anti-inflammatory therapy early in DMD has the potential to reshape the long-term trajectory of the disease. The CHMP's positive opinion reflects the strength of the AGAMREE clinical and safety data and recognizes the need for better tolerated treatment options in young children. We remain committed to providing clinicians and families with a treatment option for this vulnerable population.*"

About AGAMREE® (vamorolone)

AGAMREE is a dissociative corticosteroid approved for the treatment of Duchenne muscular dystrophy (DMD). It binds selectively to the glucocorticoid receptor and triggers anti-inflammatory activity through inhibition of NF-κB-mediated gene transcription, while inducing reduced transactivation of other genes[1]. AGAMREE is not a substrate for 11-β-hydroxysteroid dehydrogenase (11β-HSD) enzymes, which are involved in the local amplification of glucocorticoid activity in tissues and have been implicated in corticosteroid-associated toxicity[2,3]. This pharmacological profile is the basis for its classification as a dissociative corticosteroid, designed to preserve anti-inflammatory efficacy while reducing the systemic effects associated with long-term conventional corticosteroid therapy[1–3].

In the pivotal Phase 2b VISION-DMD study, AGAMREE met its primary endpoint, demonstrating a statistically significant improvement in Time to Stand (TTSTAND) velocity versus placebo at 24 weeks ($p = 0.002$)[4]. The most commonly reported adverse reactions were cushingoid features, vomiting, weight increase, increased appetite, and irritability; most were mild to moderate in severity[1].

Long-term data from up to eight years of AGAMREE treatment were presented at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference in March 2026[5,6]. In propensity-matched analyses, AGAMREE demonstrated durable efficacy comparable to standard-of-care corticosteroids and a differentiated safety profile: a lower incidence of vertebral fractures versus deflazacort-treated cohorts (8.1% vs 41.9%; $p = 0.0082$)[5]; maintained normal growth trajectory with a mean height advantage of

12.17 cm versus conventional corticosteroids ($p < 0.0001$)[5,6], and a lower incidence of cataracts versus deflazacort ($p = 0.015$), with no observed cases of glaucoma[5].

▼ *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

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3. Reeves EKM, Hoffman EP, Nagaraju K, et al. VBP15: preclinical characterization of a novel anti-inflammatory delta 9,11 steroid. Bioorg Med Chem. 2013;21(8):2241–2249. Link
4. Dang UJ, Damsker JM, Guglieri M, et al. Efficacy and safety of vamorolone over 48 weeks in boys with Duchenne muscular dystrophy (VISION-DMD). Neurology. 2024;102(5):e208112. Link
5. Guglieri M, et al. Long-term impact of vamorolone on bone health compared to standard of care glucocorticoids in boys with Duchenne muscular dystrophy. Poster 62S, MDA Clinical & Scientific Conference 2026. Link
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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 Commission (EC), in the UK by the Medicines and Healthcare products Regulatory Agency (MHRA), in Switzerland by Swissmedic, in China by the National Medical Products Administration (NMPA), in Hong Kong by the Department of Health (DoH) and in Canada by Health Canada. Santhera has out-licensed the rights to AGAMREE as follows: to Catalyst Pharmaceuticals for North America; to Sperogenix Therapeutics for China and certain countries in Southeast Asia; and to Nxera Pharma for Japan, South Korea, Australia, and New Zealand. For further information, please visit www.santhera.com.

AGAMREE® is a trademark of Santhera Pharmaceuticals.

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