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Santhera Announces Orphan Drug and Priority Review Designations for AGAMREE® (Vamorolone) in South Korea

Pratteln, Switzerland, June 2, 2026 – Santhera Pharmaceuticals (SIX: SANN) today notes that AGAMREE has been granted Orphan Drug Designation (ODD) and Global Innovative Products on Fast Track (GIFT) designation by South Korea's Ministry of Food and Drug Safety (MFDS) for the treatment of Duchenne muscular dystrophy (DMD).

These designations recognize the significant unmet medical need among DMD patients in South Korea, where no alternative treatment is currently available. The GIFT designation is a priority review pathway that may shorten the standard regulatory review timeline from 120 to 90 working days, potentially accelerating access to AGAMREE for patients living with this debilitating disease. Nxera plans to submit a Marketing Authorization Application in South Korea during 2026.

Licensing partner Nxera Pharma Co., Ltd. ("Nxera") holds exclusive rights to AGAMREE for Japan, South Korea, Australia, and New Zealand under a licensing agreement entered into in January 2026. Santhera will receive sales and milestone payments and royalties on net sales of AGAMREE in the licensed territories.

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

1. AGAMREE (vamorolone) Summary of Product Characteristics. European Medicines Agency; authorised 14 December 2023. Link

2. Heier CR, Damsker JM, Yu Q, et al. VBP15, a novel anti-inflammatory and membrane-stabilizer, improves muscular dystrophy without side effects. Life Sci Alliance. 2019;2(1):e201800186. Link
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
6. McDonald CM, et al. Comparative analysis of long-term effectiveness of vamorolone versus standard of care glucocorticoid treatment in boys with Duchenne muscular dystrophy. Poster 23S, MDA Clinical & Scientific Conference 2026. 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 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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