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Santhera Notes Topline Results from Catalyst Pharmaceuticals' Phase 1 Clinical Study of AGAMREE®

Data support AGAMREE®'s potential use as a treatment across a broad range of chronic inflammatory rare diseases

Pratteln, Switzerland, July 1, 2026 – Santhera Pharmaceuticals (SIX: SANN) today notes topline results from a two-part Phase 1 clinical study of AGAMREE® conducted by its North American licensing partner, Catalyst Pharmaceuticals, Inc. ("Catalyst"). The study suggests that AGAMREE® delivers glucocorticoid and anti-inflammatory activity, while avoiding significant immunosuppressive effects, supporting its potential use as a treatment across a broad range of chronic inflammatory rare diseases.

The study, conducted in healthy adult volunteers, evaluated equipotency between deflazacort and AGAMREE® and assessed the clinical immunosuppressive potential of AGAMREE® across ascending doses. In part A, both AGAMREE® and deflazacort demonstrated expected on-target glucocorticoid receptor activity and comparable cortisol suppression at clinical doses, with AGAMREE® showing less pronounced immunosuppressive biomarker effects, consistent with the currently labelled dosing of AGAMREE® in the treatment of Duchenne muscular dystrophy (DMD). Part B demonstrated that clinically relevant immunosuppressive effects were observed only at the highest dose level, above currently approved AGAMREE® dosing. No relevant immunosuppressive effects were observed at lower dose levels.

Under an exclusive license agreement entered into in 2023, Catalyst holds commercialization rights to AGAMREE® in North America for DMD and all potential future indications, while Santhera retains a right of first negotiation for any rights outside North America relating to new indications. Under the terms of the license agreement, Santhera is eligible to receive sales-based milestone payments as well as royalties on net sales across all commercialized indications.

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 a 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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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
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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 a 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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