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AGAMREE® (vamorolone) Data Presented at MDA 2026 Confirm Comparable Long-Term Effectiveness for up to 8 Years of Treatment, with Clinically Meaningful Safety Advantages in Duchenne Muscular Dystrophy

- **Comparable long-term effectiveness to classic corticosteroids, both prednisone (p=0.8587) and deflazacort (p=0.6544), based on time to loss of ambulation**
- **80% fewer patients with vertebral fractures (8.1% vs 41.9% on deflazacort, p=0.0082)**
- **Maintenance of normal height with 12.17 cm mean height advantage vs classic corticosteroids where significant stunting was observed (p<0.0001)**
- **Cataracts in 5.3% vs 37.8% patients, significantly lower than deflazacort (p=0.015); no glaucoma observed**

Pratteln, Switzerland, March 9, 2026 – Santhera Pharmaceuticals (SIX: SANN) today announced that full results from long-term real-world comparative analyses, including baseline data from the ongoing GUARDIAN study of AGAMREE® (vamorolone), were presented at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference 2026 in Orlando, Florida.

The dataset includes up to eight years of AGAMREE exposure (median approximately five years) in boys with Duchenne muscular dystrophy (DMD). The analyses incorporate data from participants in clinical studies who continued treatment through various access programs as well as the Phase 4 GUARDIAN study (NCT06713135). Long-term data including baseline results from GUARDIAN were compared to a propensity-matched historical control cohort treated with deflazacort or prednisone.

Together, the data demonstrate that vamorolone maintains long-term effectiveness comparable to traditional corticosteroids, while improving tolerability and meaningfully reducing key steroid-associated side effects that often lead to dose reductions or discontinuations that impact long-term patient outcomes.

These data were presented during the MDA poster session on Sunday, March 8, 2026 (6:00–8:00 pm ET). In addition, Catalyst Pharmaceuticals, in conjunction with Santhera, will host an MDA Industry Forum titled “Vamorolone in DMD: Real-World Experience, Emerging Questions and the Path to Long-Term Evidence” on Tuesday, March 10, 2026, from 12:00–1:30 pm ET in the Key Largo Room, The Hilton Orlando, Orlando, FL. The forum will be presented by Craig McDonald, MD, Professor of Physical Medicine & Rehabilitation and Pediatrics, UC Davis Health.

Comparable Long-Term Effectiveness

There was no statistically significant difference in time to loss of ambulation (LoA) between pooled vamorolone doses (2–6 mg/kg/day) and classic corticosteroids (p=0.9041), or vs deflazacort (p=0.6544) and prednisone (p=0.8587) individually. The mean real-world vamorolone dose was 4.5 ± 1.8 mg/kg/day, with median exposure of approximately 5 years. These findings suggest that improved long-term safety outcomes can be achieved without compromising efficacy and that real-world dosing indicates patients may tolerate higher doses of vamorolone over the long term compared with experience using classic corticosteroids.

Maintained Growth Without Compromising Motor Function

Height trajectories were maintained on vamorolone, while growth decline and stunting were observed in classic corticosteroid cohorts as expected. Growth stunting was defined as a height z-score < –2.0, which is approximately two standard deviations below age-matched healthy boys, a commonly used threshold for clinically meaningful short stature. After 5 years, the mean height difference was +12.17 cm in favor of vamorolone. Importantly, vamorolone is the first dissociative corticosteroid to

demonstrate patients with DMD can achieve normal growth without compromising efficacy. BMI z-scores increased in both treatment groups, consistent with corticosteroid class effects.

Substantially Lower Vertebral Fracture Risk

After a median treatment duration of approximately five years, there was an 80% reduction in the proportion of patients with vertebral fractures (8.1% of vamorolone-treated boys vs 41.9% in boys treated with deflazacort, p=0.0082).

Non-vertebral fractures occurred in 27.5% of patients, including long bone fractures in 12.5%, which are at the lower end of rates reported in the literature for traditional corticosteroids, indicating a favourable profile for chronic use. Bone age remained within normal limits relative to chronological age on vamorolone, whereas delays are commonly reported with classic corticosteroids.

Favorable Long-Term Ocular and Metabolic Profile

Cataracts were observed in significantly fewer patients compared with deflazacort (5.3% vs 37.8%, p=0.015) and numerically lower vs prednisone (5.3% vs 12.1%, p=0.05). No cases of glaucoma were observed. Glucose and lipid parameters were within normal ranges in the majority of patients. Morning cortisol levels were consistent with expected adrenal suppression for corticosteroid therapy, and no new safety signals were identified.

Ongoing Data Collection

The prospective GUARDIAN study will continue to assess long-term outcomes over the coming years, including anthropometrics, muscle function, bone health, cardiac and respiratory function, eye health, pubertal development, and additional safety endpoints.

Shabir Hasham, Chief Medical Officer of Santhera, said: *"These longer-term analyses contribute to a growing body of evidence characterising the clinical profile of vamorolone as patients remain on therapy over extended periods of time. The data presented at MDA continue to indicate that vamorolone maintains effectiveness comparable to traditional corticosteroids, while showing improvements in steroid-associated safety and tolerability outcomes. As the treatment landscape for Duchenne muscular dystrophy continues to evolve, with increasing use of combination treatment strategies, understanding the long-term benefit–risk profile of corticosteroid therapy is particularly important. A treatment that preserves efficacy while reducing treatment-limiting adverse effects would be better suited to support long-term disease management, including in combination with emerging therapies. The ongoing GUARDIAN study will continue to expand our understanding of vamorolone across a broader range of real-world outcomes as patients age and remain on treatment."*

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 classic 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 classic 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 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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