

Ad hoc announcement pursuant to Art. 53 LR

## **Santhera Receives U.S. FDA Approval of AGAMREE® (vamorolone) for the Treatment of Duchenne Muscular Dystrophy**

- *Food and Drug Administration (FDA) approved AGAMREE® (vamorolone) for the treatment of Duchenne muscular dystrophy (DMD) in children and adults aged 2 years and older*
- *U.S. license holder Catalyst Pharmaceuticals plans for commercial launch in Q1-2024*
- *FDA approval triggers USD 36 million payment obligation from Catalyst to Santhera, of which USD 26 million will be used to cover Santhera's third-party milestone obligations*
- *Following the recent positive CHMP opinion on AGAMREE in DMD, approval in the European Union by the European Commission (EC) is expected in late 2023*
- *AGAMREE would then become the first drug fully approved in both the U.S. and Europe for the treatment of patients with DMD*

**Pratteln, Switzerland, October 27, 2023 – Santhera Pharmaceuticals (SIX: SANN) announces that the U.S. Food and Drug Administration (FDA) has approved AGAMREE® (vamorolone) oral suspension 40 mg/ml for the treatment of Duchenne muscular dystrophy (DMD) in patients 2 years of age and older.**

“We are delighted to secure FDA approval which comes just weeks after the positive opinion from the CHMP of the European Medicines Agency. This is a hugely important moment for DMD patients who need an efficacious and well-tolerated therapy for this debilitating condition,” said **Dario Eklund, CEO of Santhera**. “Today is a very satisfying day for the Santhera team and we are grateful to the patients, their families and physicians who participated in the vamorolone program. We look forward to working closely with our partner Catalyst Pharmaceuticals as it prepares for U.S. commercialization of the product.”

“This new drug approval gives Duchenne patients and their families one more reason for hope,” said **Pat Furlong, Founding President & CEO Parent Project Muscular Dystrophy (PPMD)**. “Steroids are considered standard of care for Duchenne, due to their valuable role in slowing disease progression. However, they often come with considerable side effects. Vamorolone has the potential to be an alternative steroid with a better tolerability profile addressing an important unmet medical need for patients.”

“I am excited for the Duchenne community who have been waiting a long time for an alternative to the current standard of care. Today's news represents the culmination of many years of research to bring vamorolone to patients,” said **Eric Hoffman, PhD, President and CEO of ReveraGen BioPharma**. “We are pleased to have worked alongside Santhera, the DMD patient community, researchers and healthcare practitioners to reach this important milestone.”

The FDA approval of AGAMREE® was based on the data from the pivotal Phase 2b VISION-DMD study as supplemented with safety information collected from three open-label studies, including extension studies. In these trials, AGAMREE was administered at doses ranging from 2 to 6 mg/kg/day, extending for a period of up to 48 months. Compared with current standard of care corticosteroids, this novel corticosteroid treatment exhibited comparable efficacy, with data suggesting a reduction in adverse events, notably related to bone health, growth trajectory and behavior. The studies in the development program were carried out by Santhera's partner ReveraGen and 32 academic clinical trial centers in 11 countries.

With the FDA approval of AGAMREE for DMD, Catalyst will pay USD 36 million to Santhera, consisting of a USD 10 million approval milestone to the Company and an additional USD 26 million to cover contracted third-party milestone obligations. Furthermore, under the terms of the agreement, Catalyst will pay Santhera sales-based milestones of up to USD 105 million as well as up to low-teen percentage royalties, and will assume Santhera's corresponding third-party royalty obligations on vamorolone sales in all indications in North America.

Santhera will now transfer the U.S. marketing authorization (NDA) for AGAMREE to its partner Catalyst Pharmaceuticals who holds an exclusive license for AGAMREE in North America and plans to launch the product in the U.S. in Q1-2024.

In Europe, following the positive opinion from the CHMP received on October 12, 2023, approval of AGAMREE (vamorolone) for the treatment of DMD by the European Commission is expected in late 2023. Subject to approval, AGAMREE will be the only medicinal product fully approved in both territories, the EU and the U.S., for the treatment of DMD.

#### **About AGAMREE® (vamorolone)**

Vamorolone is a novel drug candidate with a mode of action based on binding to the same receptor as glucocorticoids but modifying its downstream activity and is not a substrate for the 11-β-hydroxysteroid dehydrogenase (11β-HSD) enzymes that may be responsible for local tissue amplification and corticosteroid-associated toxicity in local tissues [1-3]. This mechanism has shown the potential to 'dissociate' efficacy from steroid safety concerns and therefore vamorolone is positioned as an alternative to existing corticosteroids, the current standard of care in children and adolescent patients with DMD [1-3].

In the pivotal VISION-DMD study, vamorolone 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]. The most commonly reported adverse events versus placebo from the VISION-DMD study were cushingoid features, vomiting and vitamin D deficiency. Adverse events were generally of mild to moderate severity.

Currently available data show that vamorolone, unlike corticosteroids, has no restriction of growth [4] and no negative effects on bone metabolism as demonstrated by normal bone formation and bone resorption serum markers [5].

AGAMREE (vamorolone) has Orphan Drug status for DMD in the U.S. and in Europe and has received Fast Track and Rare Pediatric Disease designations by the U.S. FDA and Promising Innovative Medicine (PIM) status from the UK MHRA for DMD. Vamorolone is not approved for use outside the United States.

References:

- [1] Guglieri M et al (2022). JAMA Neurol. 2022;79(10):1005-1014. doi:10.1001/jamaneurol.2022.2480. [Link](#).
- [2] Liu X et al (2020). Proc Natl Acad Sci USA 117:24285-24293
- [3] Heier CR et al (2019). Life Science Alliance DOI: 10.26508
- [4] Ward et al., WMS 2022, FP.27 - Poster 71. [Link](#).
- [5] 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 and pulmonary 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 by the U.S. Food and Drug Administration (FDA); in Europe, the marketing authorization application (MAA) has received a positive opinion from the European Medicines Agency's CHMP in October 2023, and is under review by the UK Medicines and Healthcare products Regulatory Agency (MHRA). Santhera has out-licensed rights to vamorolone for North America to Catalyst Pharmaceuticals and for China to Sperogenix Therapeutics. The clinical stage pipeline also includes lonodelestat to treat cystic fibrosis (CF) and other neutrophilic pulmonary diseases. For further information, please visit [www.santhera.com](http://www.santhera.com).

*AGAMREE® is a trademark of Santhera Pharmaceuticals.*

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