

Santhera Announces Approval in Canada for AGAMREE® (Vamorolone) as a Treatment for Duchenne Muscular Dystrophy

- *Health Canada approved AGAMREE for the treatment of Duchenne muscular dystrophy in patients aged 4 years and older*
- *This approval makes AGAMREE the first and only approved therapy for DMD in Canada*

Pratteln, Switzerland, October 3, 2025 – Santhera Pharmaceuticals (SIX: SANN) today notes that Health Canada has approved AGAMREE® (vamorolone) for the treatment of Duchenne muscular dystrophy (DMD) in patients aged 4 years and older following Priority Review, marking the first approved treatment for the disease in Canada. The Canadian Neuromuscular Disease registry estimates that there are more than 800 boys and young men living with DMD in Canada and coping with its debilitating symptoms.

Kye Pharmaceuticals holds exclusive Canadian commercial rights to AGAMREE for the treatment of DMD and potentially other indications, following the agreement of a sub-license with Santhera's commercialization partner Catalyst Pharmaceuticals, Inc. ("Catalyst") in July 2024. Santhera will receive royalties on Canadian sales and these sales will also contribute towards North American sales milestones payable to Santhera from its partner Catalyst.

This is the sixth independent approval by a local health authority, following positive regulatory decisions in the U.S., Europe, the UK, China and Hong Kong.

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 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 (11 β -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 existing 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 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 China by the National Medical Products Administration (NMPA) and Hong Kong by the Department of Health (DoH). Santhera has out-licensed rights to AGAMREE for North America to Catalyst Pharmaceuticals and for China and certain countries in Southeast Asia to Sperogenix Therapeutics. For further information, please visit www.santhera.com.

AGAMREE® is a trademark of Santhera Pharmaceuticals.

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About Kye Pharmaceuticals

Kye is a leading growth-stage Canadian specialty pharmaceutical company committed to bringing value to Canadians by identifying, licensing, and commercializing novel prescription medicines that may not otherwise be available to patients across Canada. With a growing pipeline of innovative medicines, Kye's portfolio spans a range of therapeutic areas including cardiology, psychiatry, pediatrics, rare diseases, neuromuscular, hematology, and neurology. Kye Pharmaceuticals is a private company, founded in Canada and focused exclusively on the healthcare needs of patients in Canada. Kye aims to provide medicines that deliver better outcomes to our partners, Canadian healthcare professionals, and most importantly, patients across Canada. Kye, headquartered in Mississauga, Ontario was recognized on the Globe & Mail 2025 Canada's Top Growing Companies list.

For more information about the company, its management, portfolio, pipeline and commitment, please visit www.kyepharm.com

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