Nxera Pharma Submits Marketing Authorization Application for Vamorolone in South Korea for the Treatment of Duchenne Muscular Dystrophy

- Submission marks an important milestone towards bringing a new treatment option to patients with DMD in South Korea.
- Vamorolone has already received South Korean Orphan Drug Designation (ODD) and Global Innovative Products on Fast Track (GIFT) designation.
- Targeting regulatory approval in South Korea in 2027 and commercial launch in 2028.

Tokyo, Japan and Cambridge, UK, 22 October 2026 – Nxera Pharma Co., Ltd. ("Nxera" or the "Company") announces that it has submitted a Marketing Authorization Application (MAA) to the Ministry of Food and Drug Safety (MFDS) in South Korea for vamorolone for the treatment of Duchenne muscular dystrophy (DMD).

As announced on 2 June 2026, vamorolone has received Orphan Drug Designation (ODD) and Global Innovative Products on Fast Track (GIFT) designation in South Korea. GIFT is a priority review pathway designed to accelerate the review of innovative medicines targeting life-threatening or serious diseases, including rare diseases, and is expected to help shorten the review timeline and support earlier patient access to vamorolone. The Company is targeting marketing authorization in South Korea in 2027, followed by a commercial launch in 2028.

In January 2026, Nxera acquired exclusive rights from Santhera Pharmaceuticals Holding ("Santhera") to develop, manufacture and commercialize vamorolone in Japan, South Korea, Australia and New Zealand. Nxera is leading regulatory approval, commercialization and manufacturing activities in these territories. Vamorolone is a dissociative corticosteroid developed to preserve anti-inflammatory efficacy while reducing the systemic side effects associated with long-term conventional corticosteroid therapy. It is approved and marketed for the treatment of DMD in the US, European Union, UK and China.

Mr. MinBok Lee, President and Representative Director of Nxera Pharma Korea, commented: " This submission marks an important milestone towards bringing a new treatment option to patients with DMD and their families in South Korea. Following the ODD and GIFT designations, we are very pleased to have reached this submission milestone as planned. DMD is a serious, progressive disease requiring long-term treatment, and we believe vamorolone has the potential to address an important unmet medical need by offering a differentiated treatment option with a more favorable long-term safety profile. We will continue to explore ways to bring vamorolone to Korean patients sooner, while working closely with the regulatory

authorities towards approval."

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 typically reduces life expectancy to before the fourth decade due to respiratory and/or cardiac failure.

About vamorolone
Vamorolone 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]. Vamorolone 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, vamorolone 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 vamorolone treatment were presented at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference in March 2026[5,6]. In propensity-matched analyses, vamorolone 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].

References
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[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.
[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.
[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.
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About Nxera Pharma
Nxera Pharma is a technology powered biopharma company in pursuit of new specialty medicines to improve the lives of patients with unmet needs in Japan and globally. The Company has built an agile, new-generation commercial business in Japan to develop and commercialize innovative medicines, including

several launched products, to address this high-value, large and growing market and those in the broader APAC region. In addition, the Company is advancing an extensive pipeline internally and in partnership with leading pharma and biotech companies powered by its unique NxWave™ GPCR structure-based drug discovery platform. Nxera Pharma operates at key locations in Tokyo and Osaka (Japan), London and Cambridge (UK), Basel (Switzerland) and Seoul (South Korea) and is listed on the Tokyo Stock Exchange (ticker: 4565).

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