National Institutes of Health
Turning Discovery Into Health

ReveraGen's Search for Safer Alternatives for Corticosteroids for Duchenne Muscular Dystrophy Reaps Stunning

Februrary 4, 2026

When you hear about AGAMREE® (vamorolone), the first drug therapy for Duchenne muscular dystrophy (DMD) approved by the FDA in October 2023, you'll be impressed not only by the drug's benefits, but also by how ReveraGen BioPharma, the drug's creator, relies on a successful patient-centered venture philanthropy business model in combination with government partners like the National Institutes of Health. This alternative therapy promises significant progress in treating muscular dystrophies, a group of rare neuromuscular genetic disorders, at a lower cost.

Unlike many other biopharmaceutical companies with traditional investors and partners, ReveraGen works for their stakeholders—the patients with DMD and their caregivers, philanthropical foundations dedicated to eradicating DMD, and government partners such as NIH and the Department of Defense Congressionally Directed Medical Research Program (CDMRP). With the help of these government funding sources, vamorolone, a first-in-class dissociative steroid with orphan designation, gained Fast-Track designation by FDA for first-in-patient studies, allowing for risk mitigation, intensive peer review, and greater innovation.

The impact of ReveraGen's technology on public health is profound. Of the 30 or so muscular dystrophies, about 30 percent are DMD, affecting males around the age of 4. Current treatment uses glucocorticoids with serious side effects that impact the heart and bones. Vamorolone offers renewed hope to children battling DMD worldwide because it is effective in improving muscle strength and slowing DMD progression, without these debilitating side effects. In fact, in animal safety studies and human phase 1 clinical trials, even with doses up to 30-times typical doses, this novel drug has almost none of the typical glucocorticoid side effects.

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Project Details from NIH RePORTER
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Leveraging NIH programs tailored for rare and neglected diseases, ReveraGen embarked on a journey marked by innovation and resilience, leveraging multiple funding programs within the NIH Small Business Program. For example, a Direct to Phase II SBIR from the National Institute of Neurological Disorders and Stroke (NINDS) supported the first studies in people which showed improvements in all measures of motor skills, and fewer side effects (i.e. vamorolone patients did not experience stunted growth the way that patients undergoing tradition treatment did). Additionally, an NINDS Phase IIB SBIR supported a pivotal clinical trial of 120 DMD boys at 31 sites in 11 . Their NIH Commercial Readiness Pilot award played a critical role in moving the product to market by supporting the development of global regulatory and intellectual property strategies, strengthening the manufacturing process, and conducting the study that supported availability to newborns. ReveraGen's success underscores the tangible impact NIH funding can have to move a new drug through the product development pipeline, from the earliest studies through later stage commercialization milestones.

The vamorolone program is anticipated to take some twelve years and cost about $80 million to become available to patients with DMD, less time and far less cost than typical for a new type of drug. Central to their pay-it-forward model is the company's commitment that two-thirds of all income from the sales and marketing of vamorolone will go back to the international nonprofit sector, allowing further investment in promising research. Reveragen continues to move forward with testing vamorolone's potential on other muscular dystrophies with NIH support - in 2025, they should complete a clinical trial to test the effectiveness of vamorolone for treating Becker Muscular Dystrophy.