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# Pfizer initiates Phase I trials for DMD

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**Currently, there are very limited options available for treating muscular dystrophy**



Pfizer recently announced that it has initiated a Phase 1b clinical trial for its mini-dystrophin gene therapy candidate, PF-06939926, in boys with Duchenne muscular dystrophy (DMD). The first boy received an infusion of the mini-dystrophin gene on March 22nd, administered under the supervision of principal investigator, Edward Smith, MD, Associate Professor of Pediatrics and Neurology at Duke University Medical Center.

Screening and enrollment of patients is expected to continue at up to four clinical research sites in the United States. Early data from this trial are expected in the first half of 2019, once all patients have been evaluated for one full year post-treatment.

“On behalf of the community of individuals and families living with Duchenne muscular dystrophy, we applaud the important step Pfizer has taken to advance a potentially transformational treatment option for boys stricken with this terrible disease,” said Debra Miller, CEO and Founder of Cure Duchenne.

The multi-center, open-label, non-randomized, ascending dose study of a single intravenous infusion of PF-06939926 will enroll approximately 12 ambulatory boys aged 5 to 12 years with DMD. In addition to evaluating safety and tolerability, the study will evaluate measurements of dystrophin expression and distribution, as well as assessments of muscle strength, quality and function.

“Investment in this trial represents the culmination of years of research on behalf of patients by scientists at Pfizer and academic medical centers, along with the support of the DMD patient advocacy community, in the important quest to advance a program that could potentially change the trajectory of this debilitating disease,” said Greg LaRosa, PhD, Senior Vice President and Chief Scientific Officer of Pfizer’s Rare Disease Research Unit. “

This clinical trial is Pfizer’s first recombinant AAV-based gene therapy program to enter the clinic stemming from Pfizer’s 2016 acquisition of Bamboo Therapeutics. Pfizer also made a recent $100 million expansion of its Sanford, North Carolina gene therapy commercial-scale manufacturing facility. Over the last several years, Pfizer has invested to create end-to-end

capabilities to design novel AAV vectors and to build capacity to manufacture gene therapy products. Pfizer is collaborating with Sangamo Therapeutics to advance gene therapy programs with an ongoing Phase 1/2 trial in Hemophilia A, and a pre-clinical program in amyotrophic lateral sclerosis (ALS). Pfizer is also collaborating with Spark Therapeutics to advance a Hemophilia B gene therapy program currently in a Phase ½.