



# Pfizer Receives FDA Fast Track Designation for Duchenne Muscular Dystrophy Investigational Gene Therapy

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NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced that its investigational gene therapy candidate (PF-06939926) being developed to treat Duchenne muscular dystrophy (DMD) received Fast Track designation from the U.S. Food and Drug Administration (FDA). PF-06939926 is currently being evaluated to determine the safety and efficacy of this gene therapy in boys with DMD.

Fast Track is a process designed to facilitate the development, and expedite the review, of new drugs that are intended to treat or prevent serious conditions that have the potential to address an unmet medical need. This designation was granted based on data from the Phase 1b study that indicated that the intravenous administration of PF-06939926 was well-tolerated during the infusion period and dystrophin expression levels were sustained over a 12-month period.

“The FDA’s decision to grant our investigational gene therapy PF-06939926 Fast Track designation underscores the urgency to address a significant unmet treatment need for Duchenne muscular dystrophy,” said Brenda Cooperstone, MD, Chief Development Officer, Rare Disease, Pfizer Global Product Development. “DMD is a devastating condition and patients, and their parents, are waiting desperately for treatment options. We are working to advance our planned Phase 3 program as quickly as possible.”

DMD is a devastating and life-threatening X-linked disease that is caused by mutations in the gene encoding dystrophin, which is needed for proper muscle membrane stability and

function. Patients present with muscle degeneration that progressively worsens with age to the extent that they require wheelchair assistance when they are in their early teens, and unfortunately, usually succumb to their disease by the time they are in their late twenties. It is estimated that there are ~10-12,000 individuals affected with DMD in the US.

#### About PF-06939926

PF-06939926 is an investigational, recombinant adeno-associated virus serotype 9 (rAAV9) capsid carrying a shortened version of the human dystrophin gene (mini-dystrophin) under the control of a human muscle-specific promoter. The rAAV9 capsid was chosen as the delivery vector because of its potential to target muscle tissue. Pfizer initiated the Phase 1b multi-center, open-label, non-randomized, ascending dose study of a single intravenous infusion of PF-06939926 in 2018. The goal of the study is to assess the safety and tolerability of this investigational gene therapy. Other objectives of the clinical study include measurement of dystrophin expression and distribution, as well as assessments of muscle strength, quality and function.

#### About Pfizer Rare Disease

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including rare hematologic, neurologic, cardiac and inherited metabolic disorders.

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

[Click here](#) to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness.

Pfizer Inc.: Breakthroughs that change patients' lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at [www.pfizer.com](http://www.pfizer.com). In addition, to learn more, please visit us on [www.pfizer.com](http://www.pfizer.com) and follow us on Twitter at @Pfizer and @Pfizer\_News, LinkedIn, YouTube and like us on Facebook at [Facebook.com/Pfizer](https://www.facebook.com/Pfizer).

**DISCLOSURE NOTICE:** The information contained in this release is as of October 1, 2020. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about PF-06939926, an investigational gene therapy to potentially treat Duchenne muscular dystrophy, including its potential benefits and a planned Phase 3 study for PF-06939926, that involve substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for our clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; the risks associated with initial and preliminary data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when regulatory authorities will approve the commencement of our planned Phase 3 study; whether and when drug applications may be filed in any jurisdictions for any potential indication for PF-06939926; whether and when any such applications may be approved by regulatory authorities, which will depend on myriad factors, including making a determination as to whether the product's benefits outweigh its known risks and determination of the product's efficacy and, if approved, whether PF-06939926 will be commercially successful; decisions by regulatory authorities impacting labeling, manufacturing processes, safety and/or other matters that

could affect the availability or commercial potential of PF-06939926; uncertainties regarding the impact of COVID-19 on our business, operations and financial results; and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2019 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results," as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at [www.sec.gov](http://www.sec.gov) and [www.pfizer.com](http://www.pfizer.com).

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