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Pfizer Doses First Participant in Phase 3 Study for Duchenne Muscular Dystrophy Investigational Gene Therapy

Thursday, January 07, 2021 - 06:45am

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced that the first participant has been dosed in the Phase 3 CIFFREO study, which will evaluate the efficacy and safety of investigational gene therapy candidate PF-06939926 in boys with Duchenne muscular dystrophy (DMD). The CIFFREO trial is expected to enroll 99 ambulatory male patients, ages 4 through 7, across 55 clinical trial sites in 15 countries. The first patient was dosed at a site in Barcelona, Spain on December 29, 2020.

CIFFREO is a Phase 3 global, multicenter, randomized, double-blind, placebo-controlled study. The primary endpoint of the study is the change from baseline in the North Star Ambulatory Assessment (NSAA) at one year. The NSAA is a 17-item test that measures gross motor function in boys with DMD. Regardless of cohort, eligible participants are scheduled to receive the investigational gene therapy, either at the start of the study or after one year following treatment with placebo. Participants will be followed in the CIFFREO study for five years after treatment with the investigational gene therapy. Trial participants will receive commercially representative drug product manufactured at Pfizer's state-of-the-art gene therapy manufacturing facility in Sanford, North Carolina.

"The initiation of our pivotal trial, which is the first Phase 3 DMD gene therapy program to begin enrolling eligible participants, is an important milestone for the community because there are currently no approved disease-modifying treatment options available for all genetic forms of DMD," said Brenda Cooperstone, MD, Chief Development Officer, Rare Disease, Pfizer Global Product Development. "We believe our gene therapy candidate, if successful in Phase 3 and approved, has the potential to significantly improve the trajectory of DMD disease progression, and we are working with worldwide regulatory authorities to initiate this program as quickly as possible in other countries."

PF-06939926 received Fast Track designation from the U.S. Food and Drug Administration in October 2020, as well as Orphan Drug and Rare Pediatric Disease designations in the United States in May 2017.

"DMD is a progressive disorder, and patients and parents are waiting desperately for treatment options," said Silvia Avila, President, Duchenne Parent Project Spain. "The initiation of this study is an important step forward for this community, and it fuels us with hope that one day there may be treatment options for boys impacted with this devastating disease."

DMD is an X-linked disease that is caused by mutations in the gene encoding dystrophin, which is needed for muscle membrane stability. Due to the lack of dystrophin, boys 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 ~140,000 boys affected with DMD worldwide and approximately 30,000 in the United States and Europe.

About CIFFREO

CIFFREO is a Phase 3 global, multi-center, randomized, double-blind, placebo-controlled study to assess the safety and efficacy of PF-06939926 investigational gene therapy in 99 ambulatory male participants, ages 4 through 7 years, with a genetic diagnosis of DMD who are on a stable daily regimen of glucocorticoids. The participants are negative for neutralizing antibodies against AAV9, as measured by the test done for the study as part of screening.

Eligible participants will be randomized into Cohort 1 or Cohort 2. Treatment will consist of two single intravenous infusions, one of PF-06939926 and one of placebo; approximately two thirds will be in Cohort 1 and receive PF-06939926 gene therapy at the start of the study and placebo after one year, and approximately one third will be in Cohort 2 and receive placebo at the start of the study and receive gene therapy after one year, if they remain eligible. All participants will be followed in an open-label extension study for 5 years after treatment with the gene therapy. The primary endpoint of the study is a change from baseline at one year in the North Star Ambulatory Assessment (NSAA) total score. For more information, visit: ciffreoduchennetrial.com.

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 promotor. The rAAV9 capsid was chosen as the delivery vector because of its potential to target muscle tissue.

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 170 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. In addition, to learn more, please visit us on

www.pfizer.com and follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

DISCLOSURE NOTICE: The information contained in this release is as of January 7, 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 Phase 3 study, 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 in other countries will approve the commencement of the Phase 3 study; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; 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 and www.pfizer.com.

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Source: Pfizer Inc.