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Pfizer to Open First U.S. Sites in Phase 3 Trial of Investigational Gene Therapy for Ambulatory Patients with Duchenne Muscular Dystrophy

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-- Regulatory authorities in United Kingdom, Canada, Taiwan, Spain and Belgium approve re-start of global Phase 3 CIFFREO study; additional reviews ongoing --

NEW YORK, NY, April 28, 2022 - Pfizer Inc. (NYSE: PFE) announced plans to open the first U.S. sites in the Phase 3 study evaluating the investigational mini-dystrophin gene therapy, fordadistrogene movaparvovec, in ambulatory patients with Duchenne muscular dystrophy (DMD). This announcement follows a notification from the U.S. Food and Drug Administration (FDA) that the agency has lifted its clinical hold on the Investigational New Drug (IND) application for fordadistrogene movaparvovec and that Pfizer has addressed the agency's requests related to the potency assay. The global Phase 3 study, CIFFREO, has been ongoing in 11 countries and was paused in December 2021 to implement a protocol amendment following a fatal serious adverse event that occurred in a Phase 1b study in the non-ambulatory cohort. To date, regulatory authorities in the United Kingdom, Canada, Taiwan, Spain and Belgium have approved the re-start of the Phase 3 study and additional global reviews are ongoing. Pending regulatory feedback, Pfizer anticipates that nearly all CIFFREO sites will open by the end of June 2022.

"Duchenne muscular dystrophy is a devastating disease with very limited treatment options, and we believe that gene therapy has the potential to significantly impact disease progression," said Brenda Cooperstone, Chief Development Officer, Rare Disease, Pfizer Global Product Development. "Pfizer is pleased to progress CIFFREO and is working as quickly as possible to activate trial sites as local regulatory and ethics approvals occur. We thank the participants in our clinical trials and their families, as well as the broader Duchenne community, for their ongoing trust and collaboration as we work to advance our investigational gene therapy."

In December 2021, a fatal serious adverse event occurred in a non-ambulatory participant in the Phase 1b study of fordadistrogene movaparvovec. Like many non-ambulatory DMD patients, the participant had more advanced disease with underlying cardiac dysfunction. Pfizer immediately paused screening, randomization and dosing in all studies of fordadistrogene movaparvovec as the independent external Data Monitoring Committee (eDMC) reviewed the data, and the U.S. FDA subsequently placed the IND on clinical hold.

Regulatory and ethics approvals to resume the CIFFREO Phase 3 ambulatory study, including the FDA's lift of its clinical hold, follow reviews of data and protocol amendments. The protocol amendments include a seven-day hospitalization period to enable close monitoring and management of patients following administration of gene therapy. In addition, Pfizer has addressed the U.S. FDA's questions related to the potency assay to enable the trial to proceed in the United States.

Pfizer recognizes the significant unmet needs for new treatment options for non-ambulatory DMD patients. The company is continuing to work with the eDMC and gene therapy experts to assess potential next steps for evaluation of fordadistrogene movaparvovec in this patient population, who are more progressed in their disease.

About Fordadistrogene Movaparvovec
DMD is caused by an absence of dystrophin, a protein that helps keep muscle cells intact. In the absence of dystrophin, muscle cells deteriorate. Fordadistrogene movaparvovec is an investigational recombinant adeno-associated virus serotype 9 (AAV9) capsid carrying a shortened version of the human dystrophin gene (mini-dystrophin) under the control of a human muscle-specific promotor. The AAV9 capsid was chosen as the delivery mechanism because of its potential to target muscle tissue.

About Duchenne Muscular Dystrophy
Duchenne muscular dystrophy (DMD) is a serious genetic disease characterized by progressive muscle degeneration and weakness. Symptoms usually manifest in early childhood between the ages of 3 and 5. The disease primarily affects boys. Muscle weakness can begin as early as age 3, first affecting the muscles of the hips, pelvic area, thighs and shoulders, and later the skeletal (voluntary) muscles in the arms, legs and trunk. By their early teens, patients typically lose their ability to walk and the heart and respiratory muscles are also affected, ultimately resulting in premature death. DMD is the most common form of muscular dystrophy worldwide with an incidence of 1 in every 3,500 to 5,000 live male births.

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.

About Pfizer: 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 April 28, 2022. 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 fordadistrogene movaparvovec, an investigational mini-dystrophin gene therapy for 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 additional regulatory authorities in other countries will approve the resumption of the Phase 3 study; whether and when the Phase 1b study for evaluation of fordadistrogene movaparvovec in the non-ambulatory patient population may resume; 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 fordadistrogene movaparvovec; 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 fordadistrogene movaparvovec 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 fordadistrogene movaparvovec; 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, 2021 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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