Pfizer Provides Update on Phase 3 Study of Investigational Gene Therapy for Ambulatory Boys with Duchenne Muscular Dystrophy

Wednesday, June 12, 2024 - 04:30pm

.q4default .bwalignc { text-align: center; list-style-position: inside }

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) today announced that CIFFREO, a Phase 3 global, multicenter, randomized, double-blind, placebo-controlled study evaluating the investigational mini-dystrophin gene therapy, fordadistrogene movaparvovec, in ambulatory patients with Duchenne muscular dystrophy (DMD) did not meet its primary endpoint of improvement in motor function among boys 4 to 7 years of age treated with the gene therapy compared to placebo. The primary endpoint in the final analysis was assessed by change in the North Star Ambulatory Assessment (NSAA) at one year after treatment. Key secondary endpoints, including 10-meter run/walk velocity and time to rise from floor velocity, also did not show a significant difference between participants treated with fordadistrogene movaparvovec and placebo.

The overall safety profile of fordadistrogene movaparvovec in the CIFFREO trial was manageable, with mostly mild to moderate adverse events, and treatment-related serious adverse events generally responding to clinical management.

“We are extremely disappointed that these results did not demonstrate the relative improvement in motor function that we had hoped. We plan to share more detailed results from the study at upcoming medical and patient advocacy meetings, with the goal of ensuring that learnings from this trial can help improve future clinical research and development of treatment options that can improve care for boys living with Duchenne muscular dystrophy,” said Dan Levy, MD, PhD, Development Head for Duchenne muscular dystrophy, Pfizer. “We are grateful for the boys, their families, advocates, and

the investigators who have participated in this research and the continuing effort to advance treatment options for this debilitating disease.”

Pfizer will continue to closely monitor all participants enrolled in the study and is evaluating appropriate next steps for the program.

About the Fordadistrogene Movaparvovec Clinical Program

CIFFREO is a Phase 3 global, multi-center, randomized, double-blind, placebo-controlled study to assess the safety and efficacy of fordadistrogene movaparvovec investigational gene therapy in ambulatory male participants, aged 4 to 7 years, with a genetic diagnosis of DMD who are on a stable daily regimen of glucocorticoids. The primary endpoint of the study is a change from baseline to one year in the North Star Ambulatory Assessment (NSAA) total score. For more information, visit ciffreoduchennetrial.com or clinicaltrials.gov.

The CIFFREO study is currently on a dosing pause due to a fatal serious adverse event in the Phase 2 DAYLIGHT trial (NCT05429372). DAYLIGHT is a study that is evaluating the safety and tolerability of fordadistrogene movaparvovec in participants 2 years to 3 years of age with DMD. Pfizer is actively working to gather additional information on the event to understand the potential cause.

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 5,000 live male births.1

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 175 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 X 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 June 12, 2024. 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, topline results from the Phase 3 CIFFREO study and plans to share more detailed results from the study at upcoming medical and patient advocacy meetings 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; uncertainties related to further analysis of data from the Phase 3 CIFFREO study, including the sharing of more detailed results from the study at upcoming medical and patient advocacy meetings; uncertainties regarding the commercial success of Pfizer's gene therapy portfolio; uncertainties regarding the impact of COVID-19 on Pfizer's 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, 2023, 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.

1 Crisafulli S, Sultana J, Fontana A, Salvo F, Messina S, Trifirò G. Global epidemiology of Duchenne muscular dystrophy: an updated systematic review and meta-analysis. Orphanet J Rare Dis. 2020;15(1):141.

Media: +1 (212) 733-1226 PfizerMediaRelations@pfizer.com Investor: +1 (212) 733-4848 IR@pfizer.com

Source: Pfizer Inc.