May 2, 2024

We are pleased to share the exciting news that the U.S. Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to fordadistrogene movaparvovec, Pfizer's investigational gene therapy in development for the treatment of Duchenne muscular dystrophy (DMD). RMAT designation is a program designed to accelerate the development and review processes for promising regenerative medicine therapies that have preliminary clinical evidence indicating that the therapy has the potential to address an unmet medical need.

We believe this designation represents an important step for the Duchenne community and for the development of fordadistrogene movaparvovec. Supported primarily by three-year findings from our Phase 1/2 trial, which were presented at the Muscular Dystrophy Association (MDA) meeting in March, RMAT designation will enable timely discussions with the FDA regarding our potential future submission as we continue our work to deliver this treatment to patients as quickly and safely as possible. The acceptance adds to the previous FDA regulatory designations such as Orphan Drug, Rare Pediatric Disease, and Fast Track designations, which acknowledge the potential of fordadistrogene movaparvovec as we await results from our randomized, placebo-controlled Phase 3 trial.

Looking ahead, our focus remains on delivering a treatment with robust clinical data to potentially support its future use. We look forward to sharing top-line results from our Phase 3 CIFFREO study in ambulatory boys 4 to 7 years of age relatively soon.

Thank you for your continued collaboration and trust as we take another step forward in advancing the development of this much-needed potential new therapy. We want to recognize the incredible dedication of the participants, their families, and the trial investigators who are contributing to the important research that supports the fordadistrogene movaparvovec program, and we look forward to sharing future updates.

Sincerely,
The Pfizer DMD gene therapy team