Parent Project Muscular Dystrophy
JOIN THE FIGHT. END DUCHENNE.

August 9, 2024

Teresa Buracchio, M.D.
Director, Office of Neuroscience, CDER, FDA

Emily Freilich, M.D.
Director, Neurology Division 1

Dear Dr. Buracchio and Dr. Freilich,

Thank you for your commitment to review potential therapies for Duchenne muscular dystrophy.

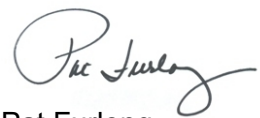
We are aware that PTC Therapeutics has recently submitted an NDA for the review of ataluren, PTC's oral small molecule treatment that allows for stop-codon read through to produce dystrophin in patients with nonsense mutations.

There are approximately 130 individuals in the United States currently on ataluren, with many of the young men on ataluren for more than 10 years. In discussions with these young men and their families, we have learned that many do not require daytime ventilation as adults, while others discuss stability in pulmonary function. As you might imagine, the worry around this compound's availability being limited or lost and experiencing a subsequent rapid decline is of great concern to these young men and their caregivers.

We commend the FDA for its commitment to appropriate regulatory flexibility and innovation in confronting the devastation of Duchenne. Our experience with earlier therapy reviews in CDER's neurology division has vividly demonstrated how patient-focused drug development programs which capture patient expectations for benefit, as well as tolerance for risk, can dramatically enable both timely advancement of individual products as well as the broader therapeutic ecosystem. This flywheel effect has helped the community advance from a treatment desert to a broadening array of options and the promise of profoundly disease-modifying treatments. The Duchenne community has partnered with the FDA and all stakeholders through groundbreaking guidance development, regulatory-grade patient preference studies, natural history studies, and a vast array of pre-competitive collaboration projects to ensure the patient voice is clearly and scientifically characterized at the center of this complex process.

I know you understand these parents and patients are in a difficult position. FDA has both the expertise and integrity to provide a full and fair review. The families who have given years to the study of ataluren would be grateful for and comforted by an FDA decision.

Sincerely,



Pat Furlong
President and CEO
Parent Project Muscular Dystrophy