December 20, 2023

Peter Marks, MD, PhD, Director, CBER
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Dear Dr. Marks:

On behalf of the Duchenne Muscular Dystrophy community, we are writing to share our views and concerns regarding the ongoing FDA review of the EMBARK trial data for ELEVIDYS. The FDA's landmark use of the Accelerated Approval process for a gene therapy to treat Duchenne has been a promising development for our families. We recognize and value how the Agency has incorporated the patient perspectives across this decision-making and encourage continued engagement as the review moves forward in an expedited and inclusive manner.

Specifically, we want to emphasize the extraordinary challenge of measuring and evaluating Duchenne disease progression in a relatively short study period. It is our belief that ELEVIDYS is demonstrating tangible and meaningful benefits for those treated when the full array of EMBARK primary and secondary endpoints data recently disclosed by the sponsor are considered. We understand that any progress against the devastating effects of Duchenne is important and certainly urge the FDA to enable access to this and all appropriate treatments for the widest possible population, informed by the totality of information available.

In addition, we acknowledge the potential risks associated with gene therapy also are a critical and evolving consideration for both regulators and, for products reaching the market, in shared decision-making by patients, caregivers, and clinicians. We believe this is achievable with a continuing commitment from product sponsors to ensure that the surveillance and analysis infrastructure exists to manage and mitigate these concerns, coupled with FDA's ongoing benefit-risk reassessments.

Our conclusion from the ELEVIDYS study data made available to the community to date, as well as our shared experiences with the relentless nature of Duchenne, is that FDA has the opportunity now to build on its record of scientific and regulatory accomplishments for confronting this disease. We urge you to pursue a transparent and timely review of ELEVIDYS using the best advice from within and outside the agency to serve the interests of people living with Duchenne Muscular Dystrophy and their families.

Sincerely,

The Best Day Ever Foundation
CureDuchenne
The Jett Foundation
The Little Hercules Foundation
Muscular Dystrophy Family Foundation
Parent Project Muscular Dystrophy
Team Joseph