VIA ELECTRONIC DELIVERY

June 14th, 2023

Robert Califf, MD, Commissioner, FDA
Peter Marks, MD, PhD, Director, CBER
Celia Witten, MD, PhD, Acting Director, OTP and Deputy CBER Director
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Dear Drs. Califf, Marks, and Witten:

On behalf of the Duchenne Muscular Dystrophy Community, we thank the Food & Drug Administration (FDA) for hosting the Cellular, Tissue, and Gene Therapies Advisory Committee meeting on May 12, 2023. We commend the agency's efforts to include the perspectives of Duchenne Muscular Dystrophy (Duchenne) families, clinicians, and scientists in the review of SRP-9001 (delandistrogene moxeparvovec). Considering the Advisory Committee's positive vote in support of Accelerated Approval of SRP-9001 to treat people with Duchenne, we urge FDA to follow the recommendation of the Advisory Committee and approve SRP-9001 through the Accelerated Approval (AA) pathway.

As expressed throughout the Advisory Committee proceedings, "time is muscle" and delays in the approval of and access to a potentially beneficial product pose a threat to patients facing Duchenne's natural history of relentless decline. Any delay means loss of muscle functionality, decreased longevity, and reduced quality of life. By contrast, skeletal muscle preservation would mean more time ambulating without aid and heart muscle preservation would mean more time without heart failure and respiratory complications. Through targeting both skeletal muscle and the heart and delivering a smaller, but functional, version of dystrophin, SRP-9001 could both improve longevity and quality of life across the stages of this devastating condition.

Therapeutic options available today for Duchenne are limited and, even considering the advances made with prior product approvals, profound unmet patient needs persist in our community. We urge FDA to use optimal regulatory flexibility under the Accelerated Approval pathway to help address these essential needs now and provide timely access to a treatment that potentially provides unique benefits to ambulatory patients with Duchenne. Delaying an Accelerated Approval decision for the completion of the ongoing Phase III study will hinder access and thereby introduce unintended harm to patients with critical needs.

As patient advocates, we also recognize the importance of Phase III studies to further define the benefit-risk profile for differing ages and stages of Duchenne and support sponsor commitments to assure the agency that studies will be continued and completed after Accelerated Approval, and that any new information generated will be communicated rapidly to the agency. Additionally, when the EMBARK data becomes available, we encourage that there be an expedited review and consideration of a broadened label, as supported by data.

We commend the FDA for its continued commitment to appropriate regulatory flexibility; innovation in confronting rare, progressive, and deadly disorders; and in empowering patient-focused drug development programs which capture, refine, and incorporate patient experiences and preferences. We

also encourage FDA to consider that a milestone approval of SRP-9001 under the Accelerated Approval pathway would stimulate further advances in Duchenne product development and support downstream impacts that build upon, and extend, the positive impact of a growing portfolio of Duchenne therapies.

As we await the anticipated June 22nd decision. Please take into account that our community cannot afford any additional delays to this opportunity now that it is within reach for those living with Duchenne and for those diagnosed in the future. We ask FDA to thoughtfully weigh and factor in the voice and needs of the patients and of our families, and give every consideration to approve SRP-9001. We believe access to gene therapy treatments would be a major step forward for all individuals living with Duchenne and those who are soon to be diagnosed. Those living with Duchenne and their loved ones cannot wait.

Sincerely,

CureDuchenne

Parent Project Muscular Dystrophy

The Little Hercules Foundation

Team Joseph

The Muscular Dystrophy Family Foundation