February 16, 2024

Advisory Committee on Heritable Disorders in Newborns and Children
Maternal and Child Health Bureau
Division of Services for Children with Special Health Needs
Health Resources and Services Administration
5600 Fisher's Lane
Rockville, MD 20857

Dear CDR Leticia Manning and Members of the Advisory Committee on Heritable Disorders in Newborns and Children:

We hope this letter finds you well. In our capacity as the nominators for the Duchenne package, we are writing to formally request a postponement of the anticipated May vote regarding the inclusion of Duchenne on the Recommended Uniform Screening Panel (RUSP) until the August meeting.

In the coming months, we expect several salient updates and developments in DMD to occur that should further instruct the Committee's consideration that are unlikely to occur prior to the May meeting. In addition to potential publication of sibling studies and clinical trial readouts, the Food and Drug Administration has set June 21st, 2024 as the date by which they intend to decide whether they will remove age and ambulation restrictions on the current approved gene transfer therapy, ELEVIDYS, for DMD. Consequently, incorporating these updates and data into the evidence review and subsequent consideration and vote will be vital for ensuring its comprehensiveness.

As per discussions that took place during the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) on November 3, 2023[1], it is our understanding that the Duchenne nomination package will continue to undergo review within the existing framework. Given the advanced stage of the review under the existing framework, we request that our DMD nomination be considered under the existing framework even with a postponement and ask for your confirmation that this will be the case.

[1] https://www.hrsa.gov/sites/default/files/hrsa/advisory-committees/heritable-disorders/meetings/achdnc-november-meeting-day-2.pdf

We firmly believe that deferring the May vote serves the best interests of both our community and the Committee. We eagerly anticipate our ongoing collaboration as the evidence review process unfolds. Please do not hesitate to reach out should you require further clarification or information.

Sincerely,

Paul Melmeyer, MPP
Vice President, Public Policy and Advocacy
Muscular Dystrophy Association

Pat Furlong
Founding President & CEO
Parent Project Muscular Dystrophy

CC: Jeffrey P. Brosco, M.D., Ph.D., Director, Maternal and Child Health Bureau, Division of Services for Children with Special Health Needs