December 2, 2025

Re: PDEV-19136 Discussion

Dear Members of the Application Review Subcommittee,

On behalf of the Muscular Dystrophy Association (MDA), I am writing to express enthusiastic support for funding of PDEV-19136, "IND-enabling activities for a gene editing therapy for Duchenne muscular dystrophy".

Duchenne remains a debilitating and progressive disease with no cure. Time is everything for families living with Duchenne, and we cannot afford to delay the development of new therapeutic approaches like MyoDys$^{45\text{-}55}$ which could benefit current and future generations of patients.

MDA has been an early and unwavering proponent of genetic medicines because neuromuscular diseases are largely single gene disorders. Beginning in the 1980s, MDA supported pioneering research which led to the identification of many disease-causing genes. In the 1990s, our focus shifted to the development of medicines which restore the function of these genes. Since our founding 75 years ago, MDA has invested more than $1 billion in research with more than $125M directed toward the development of genetic medicines, including gene replacement therapies, exon skipping antisense oligonucleotides, and gene editing therapies.

The MyoDys$^{45\text{-}55}$ program has received two awards through MDA's highly competitive and rigorously scientifically reviewed grant program and we are very supportive of continued development. I urge the Subcommittee to approve funding of PDEV-19136 to continue to advance this important work.

Sincerely,

Sharon E Hesterlee, PhD
President & CEO