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NORTHWESTERN
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December 2, 2025

Re: PDEV-19136 Discussion

Dear Members of the Application Review Subcommittee,

As a physician and scientist, I am keenly interested in bringing the benefits of research discovery to the practice of medicine. In the clinic, I witness first-hand the need for new and improved treatments for Duchenne muscular dystrophy. Duchenne is progressive and fatal, robbing patients of their physical independence and significantly reducing their lifespan. This outcome will only change through continued medical innovation.

Gene editing is a relatively new technology that offers advantages over existing approaches, including by permanently correcting mutations *in vivo* and by restoring expression of a nearly full-length dystrophin protein which will provide more full function for patients. This cannot be done with current gene replacement therapies that only provide less than one-third of dystrophin protein.

To tackle the challenge of Duchenne, the field must investigate innovative new approaches. I urge the Subcommittee to consider funding PDEV-19136 which would advance $\text{MyoDys}^{45\text{-}55}$, a new gene editing therapy, through late-stage preclinical development and IND submission.

Sincerely,

Elizabeth M. McNally

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