Parent Project Muscular Dystrophy

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December 5th, 2025

Re: PDEV-19136 Discussion

Dear Members of the Application Review Subcommittee,

On behalf of Parent Project Muscular Dystrophy (PPMD), the largest nonprofit organization in the United States solely focused on Duchenne muscular dystrophy, I am writing to advocate for funding of PDEV-19136, “IND-enabling activities for a gene editing therapy for Duchenne muscular dystrophy”. PPMD has invested $625,000 in the MyoDys$^{45\text{-}55}$ program, with three awards spanning discovery to late preclinical stage. The work proposed in this CIRM application would build on this investment to further advance development and provide an important treatment option to our community.

Duchenne is 100% fatal, typically leading to death in the 20-30s. New therapies for Duchenne are urgently needed and we cannot afford to delay the development of novel approaches that could benefit our boys. With no cure in hand, families are desperately waiting for the next generation of gene therapies that could correct the underlying cause of disease and slow or stop progression. Restoring highly functional dystrophin protein production in skeletal and cardiac muscle is paramount and the foundation upon which other therapeutic interventions can be layered.

MyoDys$^{45\text{-}55}$, the gene editing therapy described in this application, is designed to permanently remove the mutations responsible for half of all Duchenne cases and restore the missing dystrophin protein. Permanently restoring dystrophin production with this 45-55 deletion represents a truly transformational therapy, with a dystrophin protein that is known to be much more functional than current microdystrophin strategies with approved and investigational products. This approach, generating permanent gene correction, also addresses one of the critical shortcomings of current gene therapy approaches which is their durability and subsequent inability to re-dose patients with AAV. I strongly encourage the Committee to consider funding this important translational work, especially since CIRM does not have other advanced Duchenne projects in their portfolio.

Sincerely,

Eric Camino, PhD
Vice President, Research and Clinical Innovation
Parent Project Muscular Dystrophy