UCLA Childrens Hospital
Pediatric Cardiology

David Geffen School of Medicine

Daniel Levi, MD, Chief
Juan Alejos, MD
Christine Bui, MD
Nancy Halnon, MD
Majid Husain, MD
Jeremy Moore, MD

Claire Newlon, MD
Greg Perens, MD
Leigh Reardon, MD
Gary Satou, MD
Kevin Shannon, MD
Sonia Voleti, MD

UCLA Children's Heart Center
200 UCLA Medical Plaza, Suite 330
Los Angeles, CA 90095-1743
Office: (310) 267-7667
FAX: (310) 825-9524

December4, 2025

Re: PDEV-19136 Discussion

Dear Members of the Application Review Subcommittee,

I am a pediatric cardiologist and clinical researcher who treats and studies children with Duchenne muscular dystrophy. In this capacity, I am writing to encourage the Subcommittee to fund "IND-enabling activities for a gene editing therapy for Duchenne muscular dystrophy" (PDEV-19136).
There is a significant unmet need in Duchenne for therapies which can stabilize or stop disease progression. While recent decades have brought many important advances in care, there remains a substantial physical, psychological and financial burden for patients and their families. Affected individuals continue to experience loss of mobility, loss of independent breathing and loss of cardiac function. Cardiomyopathy in DMD is relentlessly progressive, and ultimately, fatal even when usual DMD and heart failure therapies are aggressively employed.

I am very supportive of translational research which may lead to new therapy options for Duchenne patients. Gene editing is an exciting new field that offers a unique strategy for correcting disease-causing mutations. I believe it is important for CIRM to consider funding the MyoDys[45-55] program, a highly innovative approach to the treatment of Duchenne.

Sincerely,

Nancy J. Halnon, M.D.
Health Sciences Clinical Professor
Division of Pediatric Cardiology
Center for Duchenne Muscular Dystrophy at UCLA