



NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.™



THE OHIO STATE UNIVERSITY
COLLEGE OF MEDICINE

June 23, 2026

Re: CIRM Grant Application – CLN6 Gene Therapy Program
Applicant/Sponsor: The Charlotte & Gwenyth Gray Foundation

To the California Institute for Regenerative Medicine (CIRM) Review Committee,

I am pleased to provide this letter of strong support to support the funding of the Charlotte & Gwenyth Gray Foundation's CIRM grant application to advance the CLN6 gene therapy program into its next phase of clinical development. I previously served as Principal Investigator for this program at Nationwide Children's Hospital and had the privilege of caring for and treating the initial cohort of children enrolled under this Investigational New Drug (IND).

CLN6 Batten disease is a devastating neurodegenerative disease that literally robs the light of development and quality of life of children who are unfortunate enough to have this diagnosis. CLN6 Batten disease is a devastating neurodegenerative disease that literally robs the light of development and quality of life of children who are unfortunate enough to have this diagnosis. I would like to emphasize the urgent need to proceed with funding this gene therapy program.

Through my direct clinical involvement, I witnessed firsthand both the devastating natural history of CLN6 disease and the extraordinary courage of the children and families who participated in this first-in-human gene therapy effort. CLN6 disease is relentlessly progressive, leading to rapid neurologic decline and premature death, and there are currently no disease-modifying treatment options available. I would like to emphasize the urgent need to proceed with funding this gene therapy program. The window for meaningful intervention narrows with each minute of their day. Through my direct clinical involvement, I witnessed firsthand both the devastating natural history of CLN6 disease and the extraordinary courage of the children and families who participated in this first-in-human gene therapy effort.

This gene therapy approach will be given at a higher dose in comparison to the original trial and will be a meaningful therapeutic option for children affected by CLN6 disease. I remain closely connected to many of the participating families and continue to appreciate both their hope for progress and their clear-eyed understanding of the risks and uncertainties inherent in early-phase clinical research. Their commitment reinforces the importance of advancing this program thoughtfully but without unnecessary delay.



NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.™



THE OHIO STATE UNIVERSITY
COLLEGE OF MEDICINE

Finally, I would like to express my confidence in the broader program leadership team supporting this effort. The Foundation has assembled an experienced and highly capable group across clinical development, regulatory strategy, manufacturing, and trial operations. Their collaborative and execution-focused approach strengthen my confidence that this program can continue to advance in a manner that is both scientifically rigorous and deeply respectful of the families it serves.

For children living with CLN6 disease, time is an irreplaceable resource. Every delay represents lost opportunities to intervene against a relentlessly progressive condition. I respectfully urge the review committee to reconsider this proposal for funding during the current grant cycle. Continued support for this program offers the possibility of advancing a promising therapeutic approach and, most importantly, providing hope to children and families facing a devastating diagnosis. The opportunity to accelerate this work may help give these children a chance for a longer, healthier future, and I believe it merits your strongest consideration.

Sincerely,

Emily de los Reyes, MD
Clinical Professor of Pediatrics and Neurology
The Ohio State University
Director, Batten's disease center of excellence
Division of Pediatric Neurology
Nationwide Children's Hospital