BDSRA foundation
Batten Disease Support + Research + Advocacy

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June 23, 2026

Dear Members of the California Institute for Regenerative Medicine Governing Board,

Re: CLIN2-20114: Phase I/IIb Gene Transfer Clinical Trial for CLN6 Disease, Delivering the CLN6 Gene by Self-Complementary AAV9

On behalf of the Batten Disease Support, Research and Advocacy (BDSRA) Foundation and the global Batten disease community we represent, we write to express our strong support for the Charlotte and Gwenyth Gray Foundation's CLN6 gene therapy program and **respectfully urge the Board to consider the extraordinary urgency of this funding decision**.

Batten disease, or Neuronal Ceroid Lipofuscinosis (NCL), is a family of 13 genetically distinct, severe neurodegenerative disorders that primarily affect children and adolescents. CLN6 disease is one of the more prevalent forms of Batten disease, accounting for an estimated 5–10% of NCL cases worldwide[1-4]. Classical CLN6 disease is characterized by developmental regression, seizures, ataxia, progressive motor decline, cognitive impairment, speech and language deterioration, vision loss, and premature death, typically between five and twelve years of age[5].

The prognosis for affected children is devastating. CLN6 disease is relentlessly progressive, has no cure, and currently has no approved disease-modifying therapy. Management remains limited to symptomatic and palliative care, representing a profound and urgent unmet medical need.

The Gray Foundation CLN6 gene therapy program is not seeking funding to explore a scientific possibility. **This is a program that has already completed the scientific, manufacturing, regulatory, and clinical groundwork necessary to begin clinical trial.**

We understand that the study has FDA authorization to proceed, is actively registered on ClinicalTrials.gov[6], has manufactured clinical-grade vector available for use, has identified potential participants, and has secured the support of experienced investigators involved in the original clinical development of the program, including Dr Emily de los Reyes, MD at Nationwide Children's Hospital. The study therefore represents **a rare convergence of scientific readiness, regulatory alignment, manufacturing capability, clinical expertise, and identified participants**.

The program is ready to begin. At this stage, the principal barrier is no longer scientific feasibility, regulatory readiness, or manufacturing capability - it is funding.

We recognize and respect the rigorous scientific review process undertaken by CIRM and appreciate the concerns raised during evaluation of the original application. However, we understand substantial progress has been made since submission, including completion of the manufacturing, analytical, comparability, potency, and release-testing activities identified as areas requiring further development. **We respectfully encourage the Board to consider the program as it exists today, rather than as it existed at the time of application.**

We are deeply concerned by the notion that a future funding cycle may adequately address the current need. From an administrative perspective, a recommendation to resubmit in a future round may appear reasonable. From the perspective of children living with CLN6 disease, it is anything but.

Should this application be deferred to a future submission cycle, funding may not become available until well into 2027. For children affected by CLN6 disease, this is not a neutral delay.

While the potential impact of this program extends far beyond the initial study population, its successful initiation depends upon children who currently remain within a critical therapeutic window. Many are ambulatory today, retaining skills and neurological function that this investigational therapy seeks to preserve. However, CLN6 disease progression continues irrespective of funding cycles, review processes, or administrative timelines.

A six-month delay may result in irreversible neurological decline and loss of trial eligibility for some children who could participate today.

The urgency before the Board is therefore not simply about funding a program. It is about enabling a narrowing window of opportunity that cannot be recovered once lost.

There is another aspect of this program that we believe deserves the Board's consideration.

Unlike many therapeutic development programs supported by large commercial sponsors, the CLN6 gene therapy program has been carried forward, in large part, by families themselves.

For years, the Charlotte and Gwenyth Gray Foundation, together with CLN6 families and supporters around the world, has shouldered much of the financial burden required to initiate development and advance this therapy. Through years of fundraising, philanthropy, and extraordinary personal sacrifice, families have helped sustain a program that otherwise may never have reached this point.

Importantly, many families continue to contribute despite knowing there is no guarantee their own child will ultimately participate in, or benefit from, the study.

Today, families are once again being asked to help fund the next phase of development – not solely for their own children, but for future generations of children affected by CLN6 disease. They continue to contribute because they understand that if this program stalls, opportunities may be lost not only for current patients, but for every child diagnosed in the years ahead.

Should this burden continue to fall upon families alone?

Recent data from BDSRA Foundation's international caregiver survey in another late-infantile onset Batten disease, CLN2 disease, demonstrated the extraordinary psychosocial, practical, and financial toll associated with Batten disease[7]. Financial strain was identified as one of the most significant burdens experienced by families, alongside the relentless caregiving demands and emotional impact of witnessing their child's progressive decline.

Yet despite these challenges, families in the global CLN6 disease community continue to give their time, resources, energy, and hope in pursuit of treatments that may alter the future for others, if not their own children.

As an organization that works closely with Batten disease families across North America and around the world, the BDSRA Foundation can state unequivocally that the need for effective therapies has never been greater.

Indeed, the urgent need for **gene-targeted therapies for Batten disease has been identified as the highest-ranked research priority** by the Batten disease community. Through a large international priority setting initiative conducted by the NIH-funded Batten Disease Clinical Research Consortium, and published in *The Lancet Neurology*[8], the number one ranked priority was clear:

"What are the best gene-targeted therapy options for Batten disease, and how can this research be accelerated?"

The message from families was unequivocal. The findings demonstrated a clear and consistent preference for accelerated development of gene-targeted therapies capable of addressing the underlying cause of disease.

This conclusion was reinforced by the recent international caregiver survey in families affected by CLN2 disease. Despite the availability of a disease-modifying therapy for that subtype, 95% of caregivers reported that there remains a critical need for additional treatment options, particularly gene therapies capable of addressing the underlying cause of disease.[7]

The Charlotte and Gwenyth Gray Foundation has demonstrated **extraordinary commitment in advancing this program despite the challenges inherent to ultra-rare disease development.** What began as one family's determination to save their daughters has evolved into a significant clinical program with promising clinical data[9] and the potential to benefit children and families around the world.

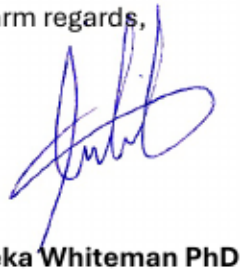
California has long been a global leader in supporting innovative therapies for patients facing the greatest unmet medical need. **We believe this CLN6 gene therapy program reflects that mission and embodies the spirit of scientific innovation, patient-centered urgency, and translational impact that CIRM was established to advance**.

On behalf of the Batten disease community, we respectfully urge the Board to give thoughtful consideration to the current readiness of this program and the unique opportunity it presents.

For many of the children currently eligible, there may not be another funding cycle.

Thank you for your time, consideration, and commitment to families affected by rare diseases.

Warm regards,



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