

To the CIRM Review Committee,

My name is Prakash Jangir, and I am writing as the father of my son, Madhav, who has been diagnosed with CLN6 Batten disease.

Madhav is currently 6 years old. Like every parent, we had dreams of watching our child grow, learn, play, and build a future. However, our family now faces the reality of a devastating neurodegenerative disease that progressively takes away a child's abilities and, ultimately, their life.

Although Madhav has already begun showing symptoms, he is still able to walk with assistance and speak a few words. Every day, we see the effects of this disease progressing, and every day matters. We know that time is not on our side.

For families affected by CLN6 Batten disease, there are currently no approved treatments that can stop or reverse the disease. The proposed scAAV9.CB.CLN6 gene therapy trial represents more than a research study—it represents hope. Hope that children like Madhav may have a chance at a better future, more time with their families, and the possibility of slowing the progression of this devastating condition.

We understand that clinical research involves risks and uncertainty. We do not ask for guarantees. We simply ask for the opportunity to pursue a potential treatment where none currently exists. Without continued research and clinical trials, families like ours are left with no options while we watch this disease progress.

We live in India, but we are fully willing to travel internationally for screening, participation, and follow-up if given the opportunity. Our family is committed to doing everything possible for our son.

I respectfully urge the committee to support funding for this clinical trial. Delays in funding may mean that children currently within the eligibility window could lose their opportunity to participate as the disease advances. For many families, this trial may be the only realistic chance to access a potential treatment.

Thank you for your time, consideration, and commitment to advancing therapies for rare diseases. Your decision has a profound impact on children like Madhav and families around the world who are living with CLN6 Batten disease every day.

Sincerely,

Prakash Jangir
Father of Madhav Jangir
India