

Support for The Charlotte & Gwyneth Gray Foundation CLN6 Gene Therapy Program



Jyotsna (Jo), Age 6½, Bangalore, India

Dear CIRM Review Team,

Thank you for your time and for considering The Charlotte & Gwyneth Gray Foundation's application to advance the CLN6 gene therapy program. We are Anjali and Praveen, parents of **Jyotsna ("Jo")**, our six-and-a-half-year-old daughter from Bangalore, India who is living with CLN6 Batten disease. We are deeply grateful to The Charlotte & Gwyneth Gray Foundation for the hope and leadership they have provided to families affected by Batten disease. Through their tireless efforts, they have connected families around the world, shared knowledge, and worked relentlessly to bring meaningful treatment options closer to reality. When Jo was diagnosed with CLN6 Batten disease in May 2024, our lives changed forever. Like every parent, we dream of seeing our daughter grow, learn, and experience all the opportunities life has to offer. A diagnosis like CLN6 introduces uncertainty into those dreams, but it has also strengthened our determination to pursue every possible opportunity that may help her future. Despite the challenges she faces, Jo remains cheerful, playful, affectionate, and full of life. She has an extraordinary ability to bring happiness to everyone around her. Her smile, resilience, and positive spirit inspire our family every day and remind us why we continue to fight for every opportunity that could improve her future. Jo currently receives treatment with Miglustat along with ongoing supportive care. While we are grateful for the medical options available today, we know there remains an urgent need for therapies that can help preserve quality of life and provide hope for children living with CLN6 Batten disease. For our family, the CLN6 gene therapy program represents more than a scientific initiative. It represents hope that children like Jo may have the opportunity to preserve important abilities, maintain their quality of life, and benefit from advances in research that were not available to previous generations of families facing this disease. We understand that one of the concerns raised during the review process relates to patient recruitment. As members of the global Batten disease community, we

can confidently say that there are families across the world actively seeking opportunities to participate in meaningful clinical research. The community that The Charlotte & Gwyneth Gray Foundation has helped build is engaged, informed, and eager to support programs that have the potential to make a difference for children affected by CLN6. We respectfully ask CIRM to consider not only the scientific promise of this program but also the urgent unmet need within the CLN6 community. For children living with rare neurodegenerative diseases, time is precious. Every month, every milestone, and every opportunity matters. The Charlotte & Gwyneth Gray Foundation has invested extraordinary effort in advancing this work, and we respectfully ask CIRM to partner with them by providing the support necessary to move this program into the highest funding tier. Funding this effort would not only advance promising science but also offer hope to families around the world who are waiting for meaningful treatment options. On behalf of Jo, our family, and the broader CLN6 community, thank you for your consideration, compassion, and commitment to improving the lives of children affected by rare diseases. We sincerely hope you will support this important program and help bring new possibilities to families like ours.

With sincere gratitude and hope,

Anjali & Praveen
Parents of Jyotsna (Jo)
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