Support Letter for Manuelli - CLN6 Batten Disease

Dear CIRM Review Team,

My name is Tainara Silva, and I am the mother of Manuelli, a six-year-old girl from Porto Alegre, Brazil, who was diagnosed with CLN6 Batten disease on September 23, 2025.

Before this disease entered our lives, Manuelli was a healthy, happy child. She developed normally, played, laughed, learned, and dreamed like any other little girl. She loved life and enjoyed the simple joys of childhood.

Today, our reality is very different. Since her diagnosis, we have watched this devastating disease progressively take away abilities that she once had. Manuelli now suffers from seizures, tremors, loss of coordination, and significant motor decline. She can no longer walk without assistance and has lost much of the independence she once had. Due to the progression of the disease, she has also returned to using diapers.

As parents, there are no words to describe the pain of watching our daughter lose skills that other children gain every day. Every month brings new challenges. Every lost ability reminds us that time is not on our side. We live with the fear of what this disease may take from her next.

Despite everything she faces, Manuelli continues to smile. She is brave, loving, strong, and full of life. She is not simply a diagnosis. She is a little girl who deserves the opportunity to grow up, make memories with her family, and have a future.

My husband and I dedicate our lives to caring for her. We attend therapies, medical appointments, and do everything possible to preserve her quality of life while searching for hope wherever it may exist. We refuse to give up on our daughter.

For our family, the CLN6 gene therapy program represents hope. Hope that the progression of this terrible disease can be slowed. Hope that children like Manuelli can preserve their abilities for longer. Hope that future families will not have to experience the same pain and uncertainty that we face every day.

Time is extremely important for children living with CLN6. Every month matters. Every week matters. Every day matters. Delays can mean the permanent loss of abilities that can never be regained.

We respectfully ask you to reconsider and support funding for this program. Behind every application are real children, real families, and real lives. For Manuelli, this research may represent the only opportunity to change the course of her disease.

Thank you for taking the time to hear our story and for considering the future of children living with CLN6 Batten disease.

With gratitude and hope,
Tainara Silva
Mother of Manuelli
Porto Alegre, Brazil

Additional Photos of Manuelli