TO: Members of the CIRM Board
FROM: Rebekah and Evan Lockard, Concerned Parents
RE: **Letter of Support for CLIN2 17091**
Date: January 29, 2025

Our children, Naomi and Jack, are both diagnosed with SPG50, an ultra-rare neurodegenerative disorder caused by mutations in the AP4M1 gene. Treatment for ultra-rare conditions is hard to find. We are lucky that a drug has been developed to treat children who have SPG50. Without treatment the condition robs children of their childhood by taking away their ability to move, speak, and think. It will ultimately rob them of their lives. **We beg you to consider funding gene therapy programs like CLIN2 17091 that only stand to improve the lives of children with rare diseases who are otherwise forgotten.**

As with most childhood diseases, time is of the essence if treatment will be effective. Naomi is 3.5 years old and every day gets closer to the day her body will start turning against her. She has low tone right now, but in just a few short months she will be at risk of developing high tone. This will start in her feet and work its way up her body until every part of her is paralyzed. In the near future she will lose her ability to move around independently. As she gets older, she will lose even more – the ability to play with toys she likes, the ability to eat food she enjoys, the ability to communicate with her loved ones. *She will lose the ability to enjoy her life.*

We do not have to watch her deteriorate. The treatment exists and we believe it works. We have seen first-hand the impact the drug has had on our son Jack, who was the youngest child to ever receive any intrathecal gene therapy at 5 months of age. Since his treatment in December 2023 Jack has thrived. He is two years younger than his sister but has caught up to her developmentally – and in many ways has passed her developmental age. Jack has strength, coordination, and cognition that is not seen in children with SPG50. He is making progress every day and may lead a normal life. **As parents, we know the treatment works**.

It is incredibly painful to have children with an ultra-rare and degenerative genetic condition. It is even more painful to know there is something that could be done to help them – but they will not get help that they need and deserve because of something as simple as money.

We respectfully request you recognize the urgent needs of our children. Every day that passes without funding is a day where they have suffered irreversible damage. **Please – consider the funding of a gene therapy in your funding decisions**. We believe that the research and trials underway deserve your support – not only for the families currently impacted but for the future generations who will otherwise suffer.

We hope that you will thoughtfully consider our plea and choose the path that will allow our children the future they deserve. Thank you for your time, consideration, and for all the work you do to make a meaningful impact on the lives of those living with disease.

Respectfully,
Rebekah & Evan Lockard
Parents of Naomi and Jack Lockard