Dear Members of the CIRM Board:

We are writing to you as concerned family members deeply affected by the devastating impact of a rare disease. Cade Jobsis (age 3) is diagnosed with SPG50, a rare neurodegenerative disorder caused by mutations in the AP4M1 gene. As you may know, rare diseases like SPG50 face a significant challenge in securing the attention, resources, and funding necessary to develop effective treatments. We urge you to consider the lives that could be improved through meaningful funding for gene therapy programs aimed at curing this disease.

SPG50 predominantly affects children, slowly robbing them of their mobility, independence, and, ultimately, their lives. Watching Cade lose their ability to walk, communicate, and ultimately even care for themselves is an indescribable pain and is our daily reality. Cade also suffers from seizures and has cognitive decline which are some of the cruel realities that those diagnosed with SPG50 face.

This is why I am reaching out to you because we cannot afford to let the voices of families affected by rare diseases go unheard. This could be the turning point that gives families like ours the hope we have longed for.

We request you to recognize the unique and urgent needs of children like Cade. Every day that passes without funding means another day closer to irreversible damage. We ask you not to ignore these children and to consider the funding 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 might otherwise suffer in silence.

We hope that you will stand with us as advocates for the families whose lives depend on it.

Thank you for your time, consideration, and for all the work that you do to make a meaningful impact on the lives of those living with disease.

Sincerely,

Jeffrey Thomson and David Janero
On behalf of 3-yr. old Cade and others like him