

Dear Members of the CIRM Board,

RE: Letter of support for CLIN2 17091

I am reaching out as a relative of a child diagnosed with SPG50, a rare neurodegenerative disorder caused by mutations in the AP4M1 gene. As you know, rare diseases face immense barriers in securing funding, despite the promising advancements in gene therapy that could change the trajectory of these conditions.

SPG50 is a progressive disorder that leads to severe motor impairment, cognitive decline, and seizures. The burden on affected families is immense, both emotionally and financially. However, recent advances in gene therapy have shown potential to halt or even reverse neurodegeneration in rare diseases like SPG50. With strategic funding, we could move these therapies from research to real-world treatment.

Investing in rare disease gene therapy not only impacts those currently affected but also lays the groundwork for future breakthroughs. We urge you to prioritize funding for SPG50 research—because innovation in one rare disease often paves the way for advancements in others.

Your support can drive meaningful change. We ask you to consider this urgent need in your funding decisions.

Best regards,
Adam Lockard
Uncle of Naomi Lockard