Jan 29th, 2025

Dear Members of the CIRM Board,

RE: Letter of support for CLIN2 17091

I am writing to express my strong support for the funding of the Phase 3 trial for SPG50, a groundbreaking gene therapy for SPG50, a rare neurodegenerative disease that primarily affects children. As the Head of Clinical Operations at Elpida Therapeutics, I have spent over two decades bringing novel therapies to the clinic for children affected by rare diseases. Throughout my career, I have worked tirelessly with subject matter experts and regulatory authorities to ensure that life-changing therapies are developed, tested, and ultimately made accessible to those who need them most.

I have had the privilege of working closely on this therapy, and I have seen the tremendous potential it holds. Our efforts to expand global access to this drug have included opening clinical trials across Europe; Spain, Italy, Germany, Denmark, and the UK all with the goal of providing accessible trials to patients and achieving regulatory success both in the United States and through the European Medicines Agency (EMA). In partnership with notable organizations such as LifeArc, Great Ormond Street Hospital in the UK, Telethon and Papa Giovanni Hospital in Italy, Fundación Columbus in Spain, and many others, we have worked diligently to ensure that this treatment reaches the children who need it.

To date, we have successfully treated six affected subjects, with the 7th subject scheduled for treatment next week and the 8th subject in April. These subjects are infants identified early enough to intervene and improve their long-term outcomes. These are just a few of the examples of the efforts we are achieving to making a difference in the lives of children who otherwise would have faced a progressive, debilitating condition with no hope for a cure.

The need for continued funding to advance this therapy cannot be overstated. These children are depending on us to help them and this work is too important to ignore. We are at a critical juncture in the development of SPG50, and we need the support of the CIRM Board to move forward with the Phase 3 trial, ensuring that we can bring this life-saving therapy to more patients and ultimately gain regulatory approval.

Please consider funding this important work. It is not only an opportunity to make a significant impact on the lives of these children but also to pave the way for future treatments for other rare diseases that affect children worldwide.

Thank you for your time and consideration.

Sincerely,
Souad Messahel
Souad Messahel, PhD

Head of Clinical Operations
Elpida Therapeutics
16501 Ventura Blvd. Suite 400
Encino, CA 91436