CURE CMT4J
ADVANCING TREATMENTS FOR RARE DISEASES

December 7, 2025

CIRM Board of Directors
California Institute for Regenerative Medicine
601 Gateway Blvd, Suite 400
South San Francisco, California 94080

Re: CLIN2-19061
Letter of Support for CLIN-2 Application – Phase I/II Clinical Trial for CMT4J

Dear CIRM Board of Directors,

On behalf of CureCMT4J/Talia Duff Foundation, I write to express our *unequivocal and deeply urgent* support for the **CLIN-2 application** submitted by **Elpida Therapeutics** to initiate a **Phase I/II clinical trial for Charcot Marie Tooth disease type 4J (CMT4J) with ELP-02**.

CMT4J is an ultra-rare, devastating neuromuscular/neurodegenerative disease often compared to ALS. Caused by biallelic pathogenic variants in the FIG4 gene, CMT4J can cause rapid progression of weakness. Most CMT4J patients experience wheelchair dependence by their teens or early twenties. Most will eventually lose the functional ability of their arms and hands. Further progression of disease impacts the respiratory muscles, causing respiratory failure, leading to ventilatory dependence and a greatly impacted quality of life.

As the mother of a child with CMT4J, I have witnessed, firsthand, the horrific loss caused by CMT4J. While working tirelessly over the past nine years to drive the research and funding forward for a gene therapy and other potential therapeutics, my daughter has lost the ability to walk, to hug us, to feed herself, and to breathe on her own. It is a parent's worst nightmare.

For CMT4J families, loss is not abstract—it is *daily*. Every single day without treatment is another day of nerve degeneration and muscle loss, another day of lost mobility, lost independence, and lost abilities. **CMT4J is a life-threatening, life-altering disease. There are currently no approved therapies or interventions to slow or halt progression of disease.**

We should have been in a clinical trial for these families in 2020; however, the Covid-19 pandemic set in, abruptly halting forward progression and causing the collapse of the biopharma industry that was then focused on cell and gene therapies for rare and ultra-rare diseases like CMT4J. Since then, we have been working desperately to get the program back on track and into the clinic.

We now have a trial-ready program with IND approval, Orphan Drug Designation and Rare Pediatric Disease Designation. Our program is backed by excellent preclinical work; an on-going natural history study with the financial support of CIRM with CLIN-1 funding; partnerships in expertise, led by Elpida Therapeutics and the Bespoke Gene Therapy Consortium, as well as translational development driven by experts in gene therapy, Charcot Marie Tooth diseases and ultra-rare disorders.

This moment—the opportunity to launch a Phase I/II clinical trial—is the culmination of a decade of persistence, partnership, sacrifice, and hope from a patient community with no other avenue.

Our Foundation's Commitment

As the central hub for the global CMT4J community, we commit to:

- **Patient Identification & Recruitment:** Mobilizing our registry and international network to support rapid, informed enrollment.
- **Community Education & Guidance:** Helping families understand trial expectations, safety measures, and the significance of participation.
- **Long-Term Patient Partnership:** Ensuring patient voices guide trial conduct and long-term follow-up.
- **Sustained Engagement:** Providing ongoing support, communication, and advocacy throughout the duration of the award.

All eyes are on our ultra-rare disease—our AAV9 gene therapy **serves as a model for tackling other rare, progressive neurodegenerative diseases, including the larger umbrella of more than 100 different subtypes of CMT, affecting 2-3 million people worldwide.**

I urge you to continue to support the development of our AAV9/FIG4 gene therapy into the clinic. CIRM's leadership in regenerative medicine aligns directly with the dire and immediate needs of our families.

Patients like my daughter have already lost so much. They have been waiting far too long. Thank you for your consideration and for your dedication to advancing therapies for patients with critical unmet needs.

With respect and profound hope.

Jocelyn A. Duff

CMT4J Mom; Founder and Executive Director
CureCMT4J/Talia Duff Foundation