December 6, 2025

California Institute for Regenerative Medicine
601 Gateway Blvd, Suite 400
South San Francisco, California 94080

RE: CLIN2-19061 Application

Dear CIRM Board of Directors:

On behalf of Elpida Therapeutics, our clinical investigators, Cure CMT4J, and the CMT4J community, we want to sincerely thank the Board and the Grants Working Group for the thoughtful and thorough review of our CLIN2-19061 application. We are grateful for the time, care, and scientific rigor applied to the evaluation of our Phase I/II clinical trial proposal for AAV9/FIG4 gene therapy (ELP-02). We fully appreciate the feedback provided and view it as essential guidance that will strengthen our program as we move toward clinical readiness.

We are also thrilled to have the support of the CIRM Team in recommending our CLIN2 proposal for funding. The opportunity to finally move forward with a phase I/II clinical trial for the CMT4J community would be an incredible step forward toward the development of a potentially life-saving, transformative therapeutic that is both first-in-human and first-in-class. This AAV9/FIG4 gene therapy program has been in development since 2016, overcoming major hurdles in the form of a global pandemic, the loss of a biopharma partner, a down turning biotech investor economy, and the loss of the Priority Review Voucher, all of which have greatly impacted the progress of this program.

We are grateful for both the Grants Working Group and the CIRM Team for recognizing the many strengths of our program, including:

- the unmet need in CMT4J
- the quality of our preclinical data
- the expertise of our clinical and scientific team
- the strong platform created by our ongoing natural history study.

These strengths speak to the potential of ELP-02 to meaningfully change the trajectory of this devastating disease.

At the same time, we recognize the weaknesses identified by the review panel and are committed to addressing each directly and comprehensively:

1. For **Access and Affordability**, we look forward to working with the CIRM team to develop a comprehensive roadmap to ensure broad, equitable access to diagnosis, trial participation, and ultimately the therapy. Additionally, a strategic plan addressing some of these areas includes:
   - Communication with the FDA regarding clinical trial design and the opportunity for a pivotal trial at the earliest sign
   - Patient identification and recruitment measures outlined below

2. For the **patient-reported outcomes (PRO's)**, our Phase I/II clinical protocol includes a number of these important measures, including:
   - Stanford-Binet - those aspects related to neuropsychology
   - CMT-Health Index (CHI) – CMT-specific subject reported outcome measure to capture disease burden
   - CMT Quality of Life (CMTQOL) – patient/subject-reported CMT-specific quality of life index designed to evaluate physical and social domains of children with CMT

   There are also a number of caregiver/parent-reported outcome measures:

   - PedsQL – measures physical, emotional, social and cognitive functioning, communication and worry
   - Vineland-3 – measures communication, daily living skills/abilities, socialization, and maladaptive behavior

   Additionally, we provide questionnaires to all interested participants in our natural history study. These surveys aim to identify which symptoms most impact their daily quality of life and what specific aspects of daily life are most challenging because of CMT4J. These will help guide outcome assessments as we progress through the clinical program. We are, however, open to any recommendations that could help strengthen or improve our clinical trial design.

3. For the **CMC Development Plan**, this has been rectified and, with funds from both CureCMT4J and CMTRF, a 500L batch of drug product (ELP-02) (typically 6 to 8 doses) has been manufactured at Viralgen for use in the Phase I/II clinical trial.

4. Regarding **recruitment**, we have several strategies in place to ensure an adequate number of participants in the clinical trial. **Over 40 patients/families have shown interest in our natural history study**, with the majority of patients falling in the range of our proposed clinical trial (2 to 20 years old).

   Since announcement of our natural history study and partnerships with CureCMT4J, CMTRF and CMTA, new patients now reach out more regularly. In addition, we are

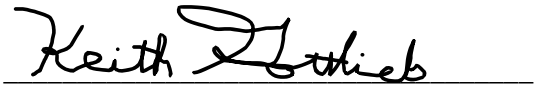
working with clinical investigators associated with the Inherited Neuropathy Consortium and CMT Centers of Excellence--with investigators across the world--to ensure patients are aware of our natural history study and proposed clinical trial for CMT4J. Other efforts already in place include:

- partnerships with genetic testing companies to inform patients/families of our work
- global patient registries through both the Inherited Neuropathy Consortium and a CMT4J-specific platform through Citizen Health
- CMT4J Working Group to address outreach to neurology and neuromuscular centers, as well as CMT Centers of Excellence
- Educational materials addressing common misdiagnoses of CMT4J

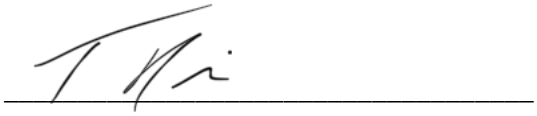
We are deeply appreciative of the CIRM process and believe the feedback has already strengthened the scientific, operational, and clinical foundation of the ELP-02 program.

Thank you again for your thoughtful review, your dedication to advancing therapies for rare diseases, and your continued leadership in regenerative medicine and gene therapy. We look forward to the opportunity to work with CIRM and together bring a transformative treatment to children and young adults living with CMT4J.

Sincerely,



Keith Gottlieb, PhD (PI)



Terry Pirovolakis, CEO and Founder

Jocelyn Duff, CMT4J Parent; Founder and Executive Director, CureCMT4J