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January 23rd, 2024

California Institute for Regenerative Medicine (CIRM)
Application Number: CLIN2-15607

Re: Support for Elpida Therapeutics' Appeal regarding CIRM application CLIN2-15607

To Whom It May Concern,

I am writing to express my strong support for Elpida Therapeutics' appeal regarding their proposal (CLIN2-15607). This project aims to conduct a Phase 3 clinical trial for Spastic Paraplegia Type 50 (SPG50).

My name is Souad Messahel, I am a Clinical Neuroscientist, and PI of the CLIN2-15607 application. I have 25 years of research experience in the drug discovery pathway, biomarker development, translational research and clinical trials. I have worked in the field of rare diseases in neurology, managing a portfolio of multiple disease programs, translational and clinical stage, both in academia and with Elpida Therapeutics.

I appeal to the ICOI to fund for our program. It is a critical program in the gene therapy space and is the most advanced in testing a platform for ultra-rare CNS disorders. I believe my application was very strong, supported by leading neuromuscular experts in the pediatric neurological field at the NIH, Boston Children's Hospital, Cedar Sinai Hospital, University of Texas Southwestern Medical Center. In addition, I received support from the Bespoke Gene Therapy Consortium (BGTC), Viralgen Viral Vector Core, Columbus Children's Foundation and AskBio Pharmaceuticals for my application.

In my application, I provided compelling evidence that this trial is set up to succeed and provide a unique opportunity to have a drug developed and approved for a rare disease indication. To date, no other CNS directed gene therapy, delivered intrathecally, has succeed to a phase 3 trial. The FDA afforded a great deal of flexibility to allow us to proceed, with a clear understanding that this will help address the severe unmet medical needs of patients with SPG50 disease. My application provided evidence from the animal models that gene therapy treatment can restore cellular function, that the longitudinal natural history study data supported the design of the

pivotal trial, and that the treatment of 4 subjects in a Phase1/2 provided evidence of drug tolerability and good safety profile. The potential impact of funding this program extends beyond the academic realm of advancing scientific knowledge but ultimately developing a targeted therapy for a rare disease. To me, it is very clear that this program aligns with the goals of CIRM as well as BGTC and serve as a pivotal study that will be a trailblazer in the gene therapy space.

I am in contact with many affected families in the US and across the world. I meet at least a new family almost every month, often within days of receiving the devastating diagnosis of their child and in some cases children. They live in hope that Elpida Therapeutics will proceed with developing a treatment for their children and begin treating individuals as soon as possible. Currently we are following 6 eligible subjects in the US and many more in Europe and the rest of the world. These children are in the critical therapeutic window for treatment and delays of the pivotal trial will mean that several if not all will fall out for inclusion. This will be a devastating outcome for them and future children not born yet especially when we have the ability to treat them now, improve their quality of life and seek approval for this treatment.

I appeal to the ICOI to fund this program. We have a unique opportunity to change the trajectory and outcome of these children and those with a future diagnosis of SPG50 disease.

Yours Sincerely,

Souad Messahel

Signature

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