



January 22, 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) evaluating the efficacy of a single dose of AAV9/AP4M1 delivered via lumbar intrathecal.

My name is Javier García, and I bring almost 30 years of expertise to the healthcare and biopharmaceutical industry. Since 2009, my focus has been on life science venture capital investing, specifically directing my efforts towards private company investments in the biopharmaceutical, life science, and medical technology sectors. I've been deeply involved in advancing viral vector technology. A significant aspect of my career centers around Viralgen, a company I co-founded, dedicated to pushing the boundaries of innovation in viral vector manufacturing for gene and cell therapies. As the president and founder of Columbus Venture Partner, my commitment to Viralgen reflects my passion for shaping the future of medicine. Moreover, as a co-founder of the Columbus Children's Foundation, I continue to contribute to transformative healthcare solutions.

As you also know our group here at Fundación Columbus is committed together with Elpida Therapeutics to treat children with rare and debilitating rare diseases.

SPG50 is an autosomal recessive disease with symptom onset in infancy resulting in delayed milestones, motor impairment, cognitive delay, progressive spasticity in limbs and quadriplegia in early adolescence. There is a serious, pressing and poignant challenge to develop treatments as the unmet need for addressing an ultra-rare disease that primarily afflicts children is significant.

The proposed Phase 3 trial, approved by the FDA, is exceptionally well designed and offers the greatest opportunity of success leading to an approved drug. I am very comfortable with the drug manufacturing, interventional and medical aspects of AAV-mediated gene transfer and its monitoring and immunological management, as well as with the outcome and efficacy measures in the proposed study.

My group at the Fundación Columbus is committed to continue support this application with advice and expertise and if successful will actively participate in the proposed clinical trial by providing support for drug manufacturing. We take immense pride in cutting edge capabilities and commitment to advancing gene therapies to the clinic. We recognize the innovative strides made by Elpida Therapeutics in their SPG50 program and support this appeal. They represent an exciting opportunity for accelerating the development and commercialization of AAV9/APM41 and we have supported their endeavor in every step of the process.

The partnership with Viralgen and Elpida Tx is expected to enable the efficient manufacture of the adeno-associated vector serotype 9 (AAV9) based therapies for use in Elpida Tx's anticipated multi-site clinical trials. Viralgen based in San Sebastian, Spain, will manufacture these therapies using its production process with the Pro10TM cell line, which is exclusively licensed from AskBio and has been found to increase scalability, performance, and yield of AAV therapies. [Viralgen/Elpida collaboration.](#)

Elpida Therapeutics is a standout force in the pharmaceutical landscape, serving as a model for others to follow. Their collaborative approach with the FDA reflects a commitment to navigating a streamlined path to drug approval, showcasing a dedication to expediting breakthrough treatments for rare diseases. What distinguishes Elpida is their commitment to transparently sharing all documents and materials with the rare disease community, creating an environment of collective learning and collaboration. Their remarkable dedication lies in instilling hope within the rare disease community, emphasizing the belief that transformative progress is possible through collective efforts. Moreover, their pledge to reinvest all profits into creating more treatments illustrates an altruistic commitment, particularly in providing support to patients facing seemingly insurmountable challenges. Elpida Therapeutics not only offers a successful blueprint but also embodies a compassionate and purpose-driven ethos, aiming to bring hope and healing to those with limited treatment options.

I support this appeal and continue to offer advice and expertise to turn this into a successful clinical trial by providing support for drug manufacturing with Viralgen.

Yours Sincerely,



Fundacion Columbus
Founder and Secretary