Columbus Children's Foundation

January 21, 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,

My name is Sheila Mikhail. With more than 20 years in biopharmaceutical leadership, I co-founded Asklepios BioPharmaceutical, Inc. (AskBio) in 2001. I served as CEO until March 2023, overseeing the establishment of three GMP manufacturing facilities in Spain and creating Viralgen and TAAV. I expanded AskBio to over 349 employees across five countries, advancing various programs into clinical trials for conditions like Pompe, late-stage Heart Failure, Parkinson's, Multiple System Atrophy, and Huntington's. In December 2020, I led the negotiation of AskBio's acquisition by Bayer AG, subsequently growing the company to over 800 employees globally. Before AskBio, I was CEO and co-founder of Bamboo Therapeutics, which was acquired by Pfizer in August 2016.

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 Hereditary Spastic Paraplegia Type 50 (SPG50).

SPG50 is a devastating disease that causes neurodevelopmental and neurodegenerative symptoms in children starting in infancy. Affected children become cognitively impaired and quadriplegic in childhood and families endure significant disease burden. There are currently no approved treatments that can change their outcome and improve their quality of life and early intervention is critical to prevent disease progressing.

I have supported the development of SPG50 gene therapy program from its early stages and subsequently the formation of Elpida Therapeutics, believing this to be a critical endeavor in addressing the unmet medical needs of these patients. My personal experience with Elpida Therapeutics over the last 12 months has been rewarding, their ambitious commitment and extraordinary progress in the rare neurogenetic field is a testimony to their dedication and success.

Elpida Therapeutics stands as a beacon of innovation and compassion in the pharmaceutical landscape, providing a compelling template for others to emulate. Their collaborative approach with the FDA exemplifies a commitment to navigating a streamlined path to drug approval, demonstrating a dedication to accelerating breakthrough treatments for rare diseases. Notably, Elpida goes beyond the norm by committing to the transparent sharing of all documents and materials with the rare disease community, fostering an environment of collective learning and collaboration. What sets Elpida apart is their profound dedication to instilling hope within the

rare disease community, exemplifying the belief that transformative progress is achievable through collective efforts. Additionally, the pledge to reinvest all profits into creating more treatments exemplifies their altruistic commitment, particularly in extending a lifeline to patients facing seemingly insurmountable challenges. Elpida Therapeutics not only offers a blueprint for success but also embodies a compassionate and purpose-driven ethos that seeks to bring hope and healing to those with limited treatment options.

I ask the ICOC to consider funding this application. If we are to change the trajectory of drug development in the rare disease space forever, then supporting a pivotal trial for an ultra-rare disease is a significant milestone. To date, there are no other pivotal trials with intrathecal delivery of brain directed genetic therapies approved to proceed by the FDA. As such, CIRM's partnership to bring an approved drug to the market for an ultra-rare indication would pave a path for others to follow and lead the efforts to transform good science into great medicines.

I have faith that our appeal will be heard, and the partnership will continue.

Yours Sincerely,

Sheila Mikhail
Co-Founder and Executive Director
Columbus Children's Foundation