December 2, 2024

Dear Members of the Application Review Subcommittee,

My name is Ben Bell and my wife and I are writing to encourage your support of a cell therapy proposal led by Dr. Katelyn Masiuk at UCLA (**Hematopoietic Stem Cell Gene Therapy (HSCGT) for MPSIIIB (Sanfilippo B) Syndrome; TRAN1-16907**).

I (Ben) am a Senior Scientist at Merck & Co., Inc. in South San Francisco where I work in biologics drug discovery and development, focusing on novel protein-based therapies such as targeted chemotherapy or cell and gene therapies that harness patients' immune cells to combat disease. Additionally, I completed my Ph.D. training in Molecular and Cellular Physiology at Stanford University School of Medicine and have benefited tremendously from CIRM's generous support of Stanford over the years. CIRM is a champion and advocate for biomedical research in the state, for which I am personally and professionally grateful.

However, we are also writing as friends of a family impacted by Sanfilippo Syndrome Type B (MPSIIIB). Dan and Minnie Lau discovered earlier this year that their youngest son, Matthew, would experience severe neurodegeneration over the course of his childhood and ultimately die before adulthood. The happy, smiley kid that we have seen running around church with his older brother or playing and giggling with his cousins will, without extraordinary intervention, soon regress to a point where his personality is dulled, his neurological skills decline, and any ability to engage with the world is diminished. Despite a well-established biological understanding of the disease mechanism and promising therapeutic proof-of-concept from a related condition (MPSIIIA), there are currently no effective treatment options available for this fatal disease.

Cell therapies like the one proposed by Dr. Masiuk offer remarkable potential for MPSIIIB, yet such therapies are notably different from traditional small molecule drugs. We in the pharmaceutical industry are still in relatively early days for cell therapies, having seen the potential for these approaches but still in need of substantial investment for further platform development. Biomedical research is a costly enterprise that can hinder some of the most innovative ideas from being realized simply due to resources. Unfortunately, families like Matthew's, and other countless families impacted by rare genetic diseases like MPSIIIB, don't have the luxury of waiting until our industry implements cell therapy at scale. They rely on CIRM to bridge the funding gap where private industry falls short.

It is our prayer that one day we will be able to intervene quickly for children who have rare genetic diseases like Matthew with affordable, accessible, and effective treatments. CIRM is already doing extraordinary work to achieve this vision, and we urge you to fund **TRAN1-16907** to continue in this tradition. Thank you for considering this important work and all you do for Californians.

Sincerely,

Benjamin N Bell Crystal Bell

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