

January 4, 2025

Subject: Letter of Support for **TRAN1-16907**

Dear Members of the Application Review Subcommittee,

I am writing to strongly encourage the California Institute for Regenerative Medicine to provide research grant funding for the Hematopoietic Stem Cell Gene Therapy (HSCGT) for MPSIIIB (Sanfilippo B) Syndrome under the leadership of principal investigator Katelyn Masiuk M.D., Ph.D.

I live in Mountain View, California, and have a personal interest in this research because I am dear family friends of Matthew Lau, a 3 year old delight of joyfulness and gentle curiosity. Our church family has grieved Matthew's recent diagnosis of Sanfilippo Syndrome Type B, a rare disease with little to no treatment options. This fatal disease will permanently alter the Lau Family, as well as the childhood of Matthew's older brother, Nathan.

Dr. Masiuk's research proposal may preserve the life of Matthew and others like him. Its treatment is similar to the related variant Sanfilippo Type A, which has shown early positive results. Dr. Masiuk is joined by Dr. Don Kohn, a pioneer in the development of lentiviral gene therapy, and Dr. Brian Bigger, a leader in the development of ex vivo lentiviral therapies for other forms of mucopolysaccharidosis, such as the program in MPSIIIA.

MPSIIIB families depend on CIRM to bridge the funding gap where private industry falls short. We strongly implore the board to fund TRAN1-16907. Please consider the expansive impact this grant will have on families in California and across the world.

Sincerely,

Janette Chan

