Subject: Letter of Support for **TRAN1-16907** Nov 2024
Hematopoietic Stem Cell Gene Therapy for MPSIIIB (Sanfilippo B) Syndrome

Dear Members of the Application Review Subcommittee,

We are 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.

We are a family of four in San Jose, California and have a personal interest in this research. Our vibrant son Matthew, age 3, is a happy, thoughtful child with a big heart for others. He saves treats for mommy, loves running around and being silly with his big brother and cousins, and prays for his grandparents nightly. He had a bright future ahead of him until he received the devastating diagnosis of Sanfilippo Syndrome Type B. In just a few years, this disease will eliminate his personality as well as all physical abilities and neurological skills, ending his life before adulthood.

We've seen that raising a child with Sanfilippo Syndrome is a heavy burden, requiring 24/7 caregiving responsibilities, frequent medical appointments (100+ for us in 2024), and healthcare expenses. The disease affects families from diverse ethnic backgrounds — Black, White, Hispanic, Asian, Arab — and across all socioeconomic classes. Lower-income families bear a disproportionate burden balancing limited time and financial constraints, and would receive an outsized benefit with the advent of this treatment.

Dr Masiuk's research proposal offers a timely lifeline for Matthew and others like him. It follows in the footsteps of a similar treatment for the related variant Sanfilippo Type A, which shows early positive results. Dr. Masiuk is joined by the world-renowned Dr. Don Kohn who is a pioneer in the development of lentiviral gene therapy, and Dr. Brian Bigger who has led the development of ex vivo lentiviral therapies for other forms of mucopolysaccharidosis, including the program in MPSIIIA. These early advances inspire confidence that Sanfilippo Type B (MPSIIIB) can be cured in the near future.

Time is of the essence for children with Sanfilippo Syndrome. We urge you to consider the profound impact this grant will have on families across our beautiful state of California and around the world.

Thank you for your dedication in advancing regenerative medicine breakthroughs. We trust that your committee will recognize the potential and provide the necessary funding to bring hope to families like ours.

Sincerely,
Daniel & Minnie Lau, parents of Matthew