Dec 2024

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

Dear Members of the Application Review Subcommittee,

We are writing to earnestly urge the California Institute for Regenerative Medicine to allocate research grant funding for the Hematopoietic Stem Cell Gene Therapy (HSCGT) for MPSIIIB (Sanfilippo B) Syndrome, led by principal investigator Dr. Katelyn Masiuk, M.D., Ph.D.

We are writing to you on behalf of a very dear friend of ours - Matthew, a sweet and genuine 3-year-old boy. Despite his young age, Matthew faces an enormous challenge—he is living with Sanfilippo Syndrome.

Matthew is a curious, kind-hearted boy who loves trains, vehicles, and has the world's best hair. He has a precious smile and we recently taught him to say that my husband is his best friend. However, in a few years, this curious, sweet, and vibrant Matthew that we know will cease to exist. This disease will gradually strip away his personality, physical abilities, and neurological functions, ultimately leading to the loss of his life before reaching adulthood.

We've also seen Matthew's diagnosis change not only his own life, but his family's as well. There is the eventual 24/7 hour care that will be needed, but even this year, there are many appointments each week (over 100 in 2024), as well as the financial burden that our friends bear.

Dr. Masiuk's research proposal brings hope for Matthew and others facing similar challenges. Building on the success of a comparable treatment for Sanfilippo Type A, which has shown promising early results, this proposal is supported by a team of exceptional experts. Dr. Masiuk is collaborating with the esteemed Dr. Don Kohn, a trailblazer in lentiviral gene therapy, and Dr. Brian Bigger, who has been instrumental in developing ex vivo lentiviral therapies for various forms of mucopolysaccharidosis, including MPSIIIA. These pioneering advancements provide strong optimism that a cure for Sanfilippo Type B (MPSIIIB) is within reach.

We are reaching out to CIRM because we know the institute is a leader in advancing regenerative medicine and offering hope to families like Matthew's. Approving and funding this therapy could be a life-changing step, not just for Matthew but for countless children and families in similar situations.

Matthew is not just a statistic; he is a vibrant, sweet little boy who deserves the chance to live a full and joyful life. His family—and all who love him—are asking for your help to make this possible.

Sincerely,
Jen & Justin To, friends of Matthew Lau