

December 6, 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 Alexander and Judy Lee, of Los Gatos, California, and we are friends of a California family affected by MPSIIIB (Sanfilippo Syndrome Type B). As friends to this family, we have witnessed the extensive toll a diagnosis like Sanfilippo can take on parents and children. We are writing to encourage the funding for TRAN1-16907 led by principal investigator Katelyn Masiuk, MD, PhD.

Our children, Noah and Kayla, love to play trains and laugh with their cheerful friend, Matthew, who was diagnosed on March 20, 2024 with Sanfilippo Syndrome Type B. Our family looked forward to Matthew's growth until his parents shared about his devastating diagnosis. This disease will take away Matthew's joyful personality as well as all his physical abilities and neurological skills. We were heartbroken as we supported his parents through their heavy burden. This year, they juggled more than 100 appointments, many health expenses, and multiple calls to health insurance and medical staff on top of daily care for Matthew and his older brother. Matthew's family is not alone, as this disease burdens families from diverse ethnic backgrounds and across all socioeconomic classes.

As individuals who have witnessed firsthand the toll this disease takes on individuals and their families, we urge you to consider funding this research. By doing so, you have the opportunity to give a bright future for children like Matthew and others like him. MPSIIIB families depend on CIRM to bridge the funding gap where private industry falls short. Thank you for your consideration of TRAN1-16907.

Sincerely,
Alexander and Judy Lee

Handwritten signatures of Alexander Lee and Judy Lee. The signature on the left is 'Alexander Lee' and the signature on the right is 'Judy Lee'.