

Date: Dec 4, 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 Nelson and Joyce Chau of Austin, Texas, and we are friends of a California family, Dan and Minnie Lau, whose child Matthew is affected by MPSIIIB (Sanfilippo Syndrome Type B). We are writing to advocate for the funding of **TRAN1-16907** led by principal investigator **Katelyn Masiuk, MD, PhD**.

When we first met Matthew, he seemed like any other child, full of energy and curiosity. However, we quickly realized that although he was the same age as our daughter, he was significantly shorter and lagging in speech development (photo on right). When we heard Matthew's diagnosis, a deep sense of devastation came over us. I could see the anguish in Dan's eyes, reflecting the heartbreak he and Minnie felt knowing that the average life expectancy for children like Matthew is around 15 years. For us, the thought of their beloved son slowly losing his personality and cognitive abilities over time is almost unbearable, which makes the urgency for research and a potential cure even more significant. The Lau's, and many other families impacted by this disease, are truly deserving of our support and assistance in their fight for a better future for Matthew and others like him.



As we understand, recent studies on Sanfilippo Type A have revealed promising early results, which is incredibly encouraging. These findings offer significant hope to the wider community affected by the other Sanfilippo variants, including Sanfilippo Type B, the diagnosis that Matthew faces. I urge you to consider the profound impact that your funding can have. Together, we can change the narrative for Sanfilippo Syndrome and bring hope to those who need it most.

Thank you for your attention to this matter. We look forward to your consideration.

Sincerely,

Nelson Chau *Joyce Chau*

Nelson and Joyce Chau