

December 3, 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,

My name is Ally and I am a resident of Mountain View, CA. I am writing in support of the grant proposal submitted to the California Institute for Regenerative Medicine to fund the **Hematopoietic Stem Cell Gene Therapy (HSCGT) for MPSIIIB (Sanfilippo B) Syndrome** research program, **TRAN1-16907**.

Over the past 3 years I have had the privilege of seeing Matthew, the son of my friends Daniel and Minnie, grow into a sweet and spirited 3-year-old boy. Matthew was diagnosed earlier this year with Sanfilippo Syndrome Type B. I have personally witnessed the difficulties his parents have faced, from financial responsibilities to frequent health scares, not to mention the burden of knowledge that Matthew's physical and neurological development will soon begin regressing and that he will likely not survive into adulthood. It breaks my heart that this disease is terminal, and that for most of his already short life Matthew will suffer from seizures, movement disorders, and all sorts of development hindrances.

However, it gives me immense hope to know that there have been promising developments in gene therapy research that could potentially lead to a cure in the near future. TRAN1-16907 could result in breakthroughs that could save Matthew's life here in California and many more beyond it. I implore the committee to strongly consider providing funding for the research team behind this project in order to allow that to happen. Thank you so much for your time and consideration for Matthew's story.

Sincerely,

Ally Fang

A handwritten signature in black ink, appearing to be the name 'Ally Fang', written in a cursive style.