

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

Jan 2025

Dear Members of the Application Review Subcommittee,

We are the Chu Family (Henry, Angela, Hanna, Caleb) from Marin County and are close friends of the Lau family, who are raising a 3 year old son, Matthew, with MPSIIIB (Sanfilippo Type B). We are writing to support the fund for TRAN1-16907.

As a Senior Healthcare Administrator, I have seen the impact of how new drugs and treatments can drastically change the outcomes for a family like this. It has only been through research and advancements like these that patients who were previously hopeless have hope. We are strongly supporting 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 principal investigator Katelyn Masiuk M.D., Ph.D.

Our family has grown with the Lau family, and Matthew has been with us every step of the way, from his infectious smile, his abounding curiosity, his silliness to his friends in our children, and his extraordinary heart for those around him. When we heard of his diagnosis, we were truly heartbroken to hear how his precious life would be shortened and how little could be done to help him. Even thinking of it now, without the availability of a treatment, brings tears to our eyes. We pray for Matthew regularly and for research opportunities, like Dr. Masiuk's, that would offer hope for him and those like him.



(Matthew)

It is our hope that children like Matthew will be given the chance to experience the fullness of life. Without this research, he will lose his ability to speak, to walk, to go to school, to grow up. We hope the subcommittee will take our words to heart to prioritize the importance and immediateness of this vital work. Thank you for your consideration.

Sincerely,

Handwritten signatures of Henry and Angela Chu. The signature on the left is 'Henry' and the signature on the right is 'Angela'.

Henry & Angela Chu