

From: Huifang Jia <huifang.sally@gmail.com>

Date: Friday, December 6, 2024 at 11:55 PM

To: Claudette Mandac <cmmandac@cirm.ca.gov>, Scott Tocher <stocher@cirm.ca.gov>

Subject: [EXT] Letter to support research project for Sanfilippo Syndrome B

Dec 4, 2024

Subject: Letter of Support for **TRAN1-16907**

Dear Members of the Application Review Subcommittee,

I am Huifang Jia from Los Altos , California, and a friend of a California family impacted by MPS-III B. I am writing to support the funding for TRAN1-16907, Hematopoietic Stem Cell Gene Therapy for MPSIII B (Sanfilippo B) Syndrome. As a registered nurse, I've witnessed the extensive toll a diagnosis like Sanfilippo can take on families.

I have known Matthew Lau for almost three years watching him growing up from a little baby. He is a kid full of light that can shine upon anyone he encounters with his big smile and his gentle manner. I was shocked and deeply saddened when I heard about him being diagnosed with Sanfilippo syndrome Type B, a neurodegenerative condition that eats away the essential skills he worked hard to gain as a child. He would endure frequent seizures and movement disorders, and eventually lose his life in his teens. It is very hard to see a child like Matthew , a bright rising little star losing his light before even having the opportunity to grow into adult life that many may take for granted.

Raising a child with a genetic condition like Sanfilippo is a huge burden emotionally, physically and financially. It impacts not only the families involved , but also the community in large with the frequent medical visits , extensive care needs, loss of productivity of adults involved in care. Dr Masiuk's research for Hematopoietic Stem Cell Gene Therapy for MPSIII B (Sanfilippo B) Syndrome offers hope of life to kids like Mathew and a relief for the family and community affected.

It is my fervent hope that children like Matthew get to experience the fullness of childhood before time runs out. We hope the subcommittee will prioritize the urgency and importance of this project and take the necessary steps to support it. Thank you for your consideration.

Sincerely,

Huifang Jia,

Friend of Mathew Lau and his family