

November 27, 2024

Subject: Letter of Support for **TRAN1-16907**  
Hematopoietic Stem Cell Gene Therapy for MPSIIIB (Sanfilippo B) Syndrome  
PI: Katelyn Masiuk M.D., Ph.D

Dear Members of the Application Review Subcommittee,

My name is Jeff Chang, and I reside in northern California with my wife Eleanor and our two children. We are friends of Matthew Lau, a 3-year-old California-based patient with MPSIIIB (Sanfilippo Type B) Syndrome. I'm writing to encourage support for the grant proposal TRAN1-16907, which seeks to develop a hematopoietic stem cell gene therapy for this debilitating condition.

Sanfilippo Syndrome causes severe neurological and physical decline beginning in the early childhood years. Symptoms include progressive loss of speech, motor skills, and cognitive function. As the disease progresses, it takes an extensive toll on families and caregivers. The condition leads to early death, usually in the second or third decade of life.

Our son is the same age as Matthew, and our daughter is the same age as Matthew's older brother. We could not even imagine what it would be like for our daughter to lose her brother before he turned twenty, as likely will be the case for Matthew if a treatment option for Sanfilippo Type B is not found.

As a rare disease, the funding environment has been challenging. Several hopeful companies have gone bankrupt or abandoned plans for Sanfilippo Type B.

I strongly encourage the funding of this research and its life-changing potential for these children and their families.

Sincerely,  
Jeff and Eleanor Chang

The image shows two handwritten signatures in black ink. The signature on the left is 'Jeff Chang' and the signature on the right is 'Eleanor Y. Chang'. Both signatures are written in a cursive, flowing style.