

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 David and Charlene Liu, of Lafayette, California, and we are friends of a California family impacted by MPSIIIB (Sanfilippo Syndrome Type B). We are writing to strongly encourage the funding for TRAN1-16907 led by principal investigator, Katelyn Masiuk, MD, PhD.

We have a decades-long cherished relationship with Daniel and Minnie, and have witnessed their joyous welcome of their adorable second son, Matthew, into their lives. We remember meeting and holding Matthew in our arms less than 2 weeks after he was born, witnessing his eyes light up in curiosity at all of his family and friends and birthday cake during his 1st birthday party, and receiving grapes that he insisted on sharing as he sat eating right next to us at the dinner table. It has been and continues to be heartwarming watching Matthew grow up under the most devoted care of his parents, imitating their joyfulness and thoughtfulness towards others as well as forming his own familial care towards his older brother and family members.

It was therefore equally heartbreaking and terrifying to hear the news of his Sanfilippo Syndrome Type B diagnosis earlier this year, along with periodic news of Matthew experiencing seizures. It also weighs heavily on our hearts to see that this diagnosis has impacted our dear friends, Daniel and Minnie, in their day-to-day lives, emotionally and physically, as they balance tailored caregiving responsibilities and medical appointments, and strive hard to find an effective and life-saving treatment for their precious son.

The news of a research proposal submitted to CIRM seems to be the first real window of opportunity for a cure for Matthew. We urge you to consider funding this research, as it is our fervent hope that children like Matthew get to experience the fullness of childhood and adulthood. This is an opportunity to change the future for patients like Matthew and others like him. Thank you for your consideration of TRAN1-16907.

Sincerely,
David & Charlene Liu

Handwritten signatures of David and Charlene Liu. The signature for David is on the left, and the signature for Charlene is on the right, enclosed in a circle.