From: Debbie Yu <deborahjyu@outlook.com> Date: Monday, December 2, 2024 at 12:08 AM

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

Subject: [EXT] Letter of Support for TRAN1-16907

Dear Members of the Application Review Subcommittee,

I'm Deborah Yu, of Palo Alto, California, and I am friends with a California family whose young son is affected by MPSIIIB (Sanfilippo Syndrome Type B). I am writing to strongly encourage the funding for TRAN1-16907 led by principal investigator Katelyn Masiuk, MD, PhD.

What my friends have to endure in caring for their young son is heartbreaking. It affects a family at all levels; financial, emotional, mental, physical. Their young son, Matthew, aged 3, is not able to enjoy his childhood, a right that any child should have. Instead, he goes to hundreds of appointments in a year, with the parents shouldering the high cost. At any moment, they have to be ready to take their son to the ER, if a seizure or other critical symptom arises, all while still needing to care for the needs of the family, including Matthew's older brother.

I know time is not on our side, due to the high likelihood that Matthew will not reach adulthood. Please consider prioritizing funding this research; to provide a chance that Matthew might be able to grow up, that he might improve instead of decline as he grows up. Please help provide hope to MPSIIIB families and their friends and families that are looking for any way to help their children.

Sincerely, Deborah Yu