

12/16/2024

Dear Members of the Application Review Subcommittee,

We are Richard and Michelle Mu from San Jose, California, and we are friends of Dan and Minnie Lau, whose son Matthew Lau has been diagnosed with MPSIIB (Sanfilippo Syndrome Type B). We are writing to encourage the funding for TRAN1-16907 led by principal investigator Katelyn Masiuk, MD, PhD.

We will never forget the moment we heard the news. While we were overseas for a family vacation, the news of Matthew's diagnosis devastated us even as we tried to enjoy the rest of our vacation. There was not a moment that went by where we were not burdened by our thoughts for Matthew.

And since our vacation, we have heard the Lau family share during various occasions (from intimate to large public settings) about Matthew's situation. Because we go to the same church as the Lau's, our heart breaks over our dear friends who feel like family to us. We have firsthand witnessed the impact and toll it has taken on Dan and Minnie. While we appreciate the time they still make for others like ourselves, we also understand that, as a family, they have made a lot of sacrifices for Matthew. These sacrifices include multiple appointments for Matthew per week. This diagnosis has been mentally draining for our friends as they search for a treatment and seek out methods to alleviate some of his current symptoms related to this diagnosis. While we are thankful that he is continuing to thrive as an active and cheerful toddler, we are saddened that he has had many seizures and speech delays (for example) as a result of this condition.

We also have a daughter (Sophie) close to age to Matthew and a baby boy due this month. Sophie has had many play dates with Matthew and adores him. While we cannot fully relate specifically to the pains the Lau's are experiencing, we understand the emotional toil a parent has over their child's well-being. It has brought an awareness of how precious our time with our children really is. We admire how resilient Dan and Minnie have been over this situation and as any caring parent, they are doing their very best to seek treatment to save their child.

It is our fervent hope that children like Matthew get to experience the fullness of life that we all take for granted. We urge you to consider funding this research. By doing so, you have the opportunity to change the future for patients like Matthew and others like him. Thank you for your consideration of TRAN1-16907.

Sincerely,

Richard, Michelle, and Sophie Mu