January 19, 2025

Subject: Letter of Support for TRAN1-16907, Gene Therapy for MPSIIIB (Sanfilippo B) Syndrome

Dear Members of the Application Review committee,

We are Stanley Dai and Denice Chan from Alameda, California, and our nephew has diagnosed with MPSIIIB (Sanfilippo Syndrome Type B) last year. We are writing for your support for study TRAN1-16907 (led by Katelyn Masiuk, MD, PhD). As a labor and delivery nurse, I have seen the devastating loss of a child firsthand. No parent should have to endure outliving their own child, but this is the cruel reality faced by families battling MPSIIIB.

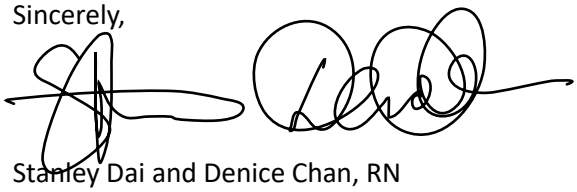
Our three-year-old nephew, Matthew is a kind, motivated boy—always eager to learn, help his brother and cousins, and brings joy to those around him. This disease is like the child's form of dementia and having seen dementia within our family, we know the anguish of losing connection with our loved ones. Watching Matthew's bright future being eclipsed by this degenerative disease is agonizing. As time is cruelly ticking away faster than it does for his peers, we know every moment matters and seize action.

If this treatment succeeds, it will give Matthew the possibility of a full life and allow him to grow up alongside his family and friends. Supporting this research could help rewrite the narrative for children with MPSIIIB, while advancing the field of neurodegenerative treatments. Your actions would inspire countless others facing similar struggles, proving that we can conquer the insurmountable with your help.

We urge you to consider funding this hopeful study. By doing so, you have the power to give children like Matthew a future they deserve—and to change the course of history for this and future families alike.

Thank you for reading and your consideration.

Sincerely,



Stanley Dai and Denice Chan, RN