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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,

We are writing to support the funding of TRAN1-16907. Brian is a managing director at a large biotechnology focused venture capital fund with no financial connection to this research and Rachel is a former Medicare healthcare policy lead. We are close friends of a MPSIIIB family in California. Together, we've witnessed the unmet needs in rare disease research, with many promising interventions struggling to progress due to the "valley of death" in funding—where early-stage innovations lack the financial support to bridge the gap between preclinical development and clinical trials and approval.

Sanfilippo Syndrome **B** (MPSIIIB) is a prime example of this funding gap. The scientific community has made significant strides in understanding the genetic and molecular basis of Sanfilippo syndrome, yet without sustained financial support, these advances remain at risk of stagnating.

Specifically, there have been several companies spanning from gene therapy to enzyme replacement therapy (e.g., Abeona, Allievex, Orchard Therapeutics, etc) that have shown promising clinical benefit in MPSIIIB. However, due to financial challenges, these companies have either delayed or wound down development of these drugs, leaving patients with no options. MPSIIIB remains a drastically underserved patient population as there are supportive care or disease modifying treatments available.

A diagnosis of Sanfilippo is burdensome on families across all income levels, particularly for those from lower-income backgrounds who lack the financial resources and flexibility to take time off work, traveling to dozens of appointments each year, while also struggling to afford the specialized care and treatments their child needs. Our friend with an MPSIIIB child has been to over 100 appointments this year. In California, with a high cost of living, these financial strains are more pronounced among lower-income families, making an already challenging situation overwhelmingly difficult for these families.

We strongly encourage the subcommittee to fund **TRAN1-16907** to close the unmet needs of MPSIIIB families.

Sincerely,



Brian Liu



Rachel Liu