

December 4, 2024

Subject: Letter of Support for **TRAN1-16907: Hematopoietic Stem Cell Gene Therapy for MPSIIIB (Sanfilippo B) Syndrome**

Dear California Institute of Regenerative Medicine,

I am Alina Gorniak M.Ed., RDN, mother to Simon who suffers from Sanfilippo type B. I am writing to ask you to approve the funds needed for TRAN1-16907, Hematopoietic Stem Cell Gene Therapy for MPSIIIB (Sanfilippo B) Syndrome.

Simon is my and my husband's one and only miracle child. Chemotherapy for a highly aggressive form of cancer was effective, yet brutal. I was left clinically infertile for two years post-treatment until our Simon surprised us; He potentially was created from the last remaining, viable egg. Simon is our everything. His smile is contagious. He is a sweet soul, and exudes a bright light.

We learned of his diagnosis when he was almost two and half years old. My husband and I were devastated. We were going to lose our only child. As a parent, I have also suffered watching this devastating disease progress in our child over the past eight years and don't want other parents or children to go through the same thing.

As someone who has seen firsthand the toll this disease takes on children and their families, I urge you to consider funding this research. By doing so, you could change the future to one of hope rather than grief for all children with Sanfilippo type B. Thank you for your consideration of TRAN1-16907.

Sincerely,

Alina Gorniak


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