

Dec 2024

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

Dear Members of the Application Review Subcommittee,

I am Cindy Ho from San Jose, CA, and a friend of a California family impacted by MPS-IIIB. I am writing to strongly encourage the California Institute for Regenerative Medicine to provide research grant funding for the Hematopoietic Stem Cell Gene Therapy (HSCGT) for MPSIIIB (Sanfilippo B) Syndrome under the leadership of principal investigator Katelyn Masiuk M.D., Ph.D. As a friend, I have seen the extensive mental and physical toll a diagnosis like Sanfilippo can take on families.

When my friends shared with me that their three year old son, Matthew was diagnosed with Sanfilippo Syndrome, my heart ached with them. I couldn't imagine seeing the bright, goofy little boy who would run up to me in a superman pose endures so much pain. Matthew has had countless doctors appointments and hospital visits, and continues to be so joyful and happy. To think that his personality will be taken away by this disease is tragic. This world would be such a brighter place if Matthew and other children with this diagnosis were cured.

Time is of the essence for children with Sanfilippo Syndrome. I urge you to consider the profound impact this grant will have on families across our beautiful state of California and around the world.

MPSIIIB families like Daniel, Minnie, and their two sons - Matthew and Nathan depend on CIRM to bridge the funding gap where private industry falls short. I strongly implore the board to fund TRAN1-16907.

Thank you for your consideration.

Sincerely,

Cindy Ho

A handwritten signature in black ink that reads "Cindy Ho". The signature is written in a cursive, flowing style.