

12/5/2024

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

Dear California Institute of Regenerative Medicine,

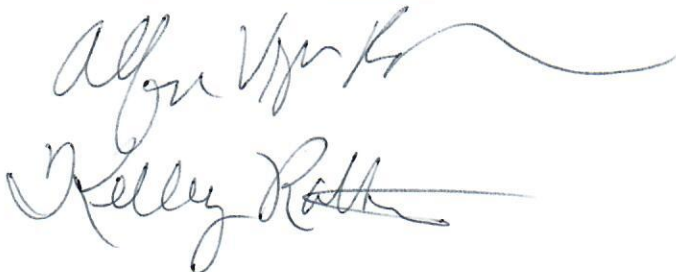
We are Dr. A. Vijay and Kelley Rathinam of Atlanta, Georgia and are parents of our beautiful daughter, Madelyn, who suffers from Sanfilippo type B. I am writing to ask you to approve the needed funds for TRAN1-16907, Hematopoietic Stem Cell Gene Therapy for MPS IIIB (Sanfilippo B) Syndrome.

As you are aware, Sanfilippo was discovered in the 1960s and it is unfortunate that 60+ years later there are still no treatments for this devastating disease. There have been lots of stops and starts in terms of research and investment in the space for many years and we are all hopeful with the scientific advancements over the last decade in the areas of gene therapy and enzyme replacement therapy, some treatment options will finally emerge. Over the years, countless children and families have suffered/perished without treatment and parents and caregivers have shepherded these children through the gut wrenching, sad and lonely journey of cognitive and physical decline. As parents, we are watching this devastating disease progress in Madelyn and don't want other parents or children to go through the same thing.

In summary, time of the essence for Madelyn and other children suffering with this terrible disease. It is our fervent hope that children like Madelyn get to experience the fullness of childhood before time runs out. We hope the funding committee will prioritize the urgency and importance of this project and take the necessary steps to support it. Thank you for your consideration.

Best regards,

Dr. A. Vijay and Kelley Rathinam

The image shows two handwritten signatures in black ink. The top signature is for Dr. A. Vijay, written in a cursive style with a long, sweeping tail. The bottom signature is for Kelley Rathinam, also in cursive, with a more compact and rounded style.