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Date: Thursday, December 5, 2024 at 6:09 PM

To: Claudette Mandac <cmadac@cirm.ca.gov>, Scott Tocher <stocher@cirm.ca.gov>

Subject: [EXT] TRAN1-16907 - December 12 Hearing - Support Letter

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December 5, 2024

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

Dear California Institute of Regenerative Medicine,

We are Roger and Susan Chapin, parents of Blair, who suffered from Sanfilippo Syndrome Type B. We are writing to urge your approval of the necessary funding for TRAN1-16907, Hematopoietic Stem Cell Gene Therapy for MPS IIIB (Sanfilippo B) Syndrome.

Blair was born seemingly perfect, with no indication of the devastating journey ahead. We had no family history or knowledge of this disease. Like any parents, we were unprepared to hear that our child had a terminal condition with no treatment or cure. The only advice we were given was to take her home and love her—which we did with all our hearts.

Despite our unwavering love and every resource we could muster, we could not shield Blair from the pain and suffering this disease inflicted. Over time, she lost her ability to speak, walk, eat, and find peace or comfort. Witnessing this decline was excruciating—not just for Blair but for our entire family.

Sanfilippo Syndrome Type B robs children of their lives and families of hope. As parents who have experienced the unimaginable toll of this disease, we implore you to fund this critical research. TRAN1-16907 offers a rare opportunity to transform a future filled with grief into one of hope for children and families impacted by this condition.

Thank you for considering this vital investment in research and the lives it can save.

Sincerely,

Roger and Susan Chapin (407-491-4499/susanchapin@icloud.com)

