

Dear Members of the Application Review Subcommittee,

I am a California taxpayer with a son with Sanfilippo B (MPS-3B) and am writing to support the work of Katelyn Masiuk, MD, PhD for Hematopoietic Stem Cell Gene Therapy for MPS-III B.

I have read through supporting materials for the Jan 30th meeting and noticed that although there are insufficient funds to meet all of the initial “recommended for funding” TRAN1 applications (≥ 85 score), there is a leftover CLIN budget of \$38.8 million as well as \$4.1 million DISC and \$2.5 million TRAN1 leftover for a total of **\$45.4 million unallocated.**

I have also read through the reviewers comments as well as PI Masiuk’s letter of response. The GWG’s vote was a **unanimously positive 13-0 on project rationale, planning and design, feasibility, and DEI principles.** The only outlier was the potential for impact, which was based on an erroneous 1:1,000,000 incidence rate. The correct incidence rate is an order of magnitude higher as highlighted in Dr Masiuk’s letter to the board.

In a disease with a high burden of care, the potential for impact is significant. The University of Florida estimates the lifetime **economic burden per patient at \$8 million¹**, totaling in the billions nationally. As we can all probably agree, the healthcare system has great need for efficiency improvements. A single patient’s reduced burden of up to \$8 million to the ecosystem (doctors, nurses, labs, administrators, etc) can potentially free up services for other types of patients without reducing the revenue incentives to providers due to the already bottlenecked pipeline of patients. The benefit to the health ecosystem extends far beyond SanfilippoB/MPS-3B.

Given the above, I **highly recommend the board to consider a motion on Jan 30th to re-allocate unused CLIN & DISC budgets for qualifying TRAN1 projects (≥ 85 score).**

Together, we can build a better future today. The power, opportunity, and responsibility is in our hands. Let us make this a reality.

Sincerely,
Daniel & Minnie Lau, parents of Matthew



References:

1. Ashby, F., Park, H., Svensson, M., Heldermon, C., (2023). Economic Burden of Sanfilippo Syndrome in the United States. <https://pmc.ncbi.nlm.nih.gov/articles/PMC10312916/>

