SCID, Angels For Life Foundation

2424 Heritage Lakes Court
Lakeland, FL 33803

SCID
Angels For Life
Foundation

August 5, 2024

To: CIRM Access and Affordability Working Group

Re: Public comment: Regular Meeting Of The Treatment and Cures Accessibility and Affordability Working Group of The California Institute For Regenerative Medicine held on August 7, 2024

I want to thank the committee for this opportunity to share my perspective. My name is Barb Ballard, and I am the Director of SCID Angels For Life Foundation, a patient advocacy group for families affected by Severe Combined Immune Deficiency or SCID.

The form of SCID I want to discuss today is Adenosine Deaminase SCID also known as ADA SCID.

The ongoing Gene Therapy trial at UCLA for ADA SCID has had a highly successful treatment rate of 35 out of 37 patients in the US, with no loss of life due to the treatment, combined with a low-intensity transplant regime. This therapy has the potential to commercialize and become widely available for equitable care of affected children.

There is currently an unmet need for this therapy. After treating those patients previously funded by CIRM, there will still be 15 children sitting on a waiting list who cannot be treated without additional funding. Additionally, more children born with ADA SCID never made it to the wait list because the outrageous wait time placed the children's lives in danger and consequently their families accepted therapies that risk a lower quality of life. Meanwhile, according to the Immune Deficiency Foundation, there are 10 children born each year with ADA SCID across the US and Canada.

To commercialize this treatment, the trial needs to be able to continue to enroll and treat patients. I implore this committee to not disregard the potential of this live-saving therapy before it can secure a road to commercialization and accessibility for all patients. I implore CIRM to approve continued funding for ADA SCID Gene Therapy.

Kind Regards,

Barb Ballard
Director
SCID Angels For Life Foundation
www.SCIDAngelsForLifeFoundation.org