

**Subject:** [EXT] Public Comment – Agenda Item 12, Support for CLIN2-20316 (In Utero Stem Cell Transplantation for Fanconi Anemia)

**Date:** Tuesday, September 22, 2026 at 5:31:40 PM Pacific Daylight Time

**From:** Matthew Goldstein

**To:** Lana Morales

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To the Application Review Subcommittee and Independent Citizens' Oversight Committee:

I am writing to urge the ARS to fund CLIN2-20316, the proposed trial of in utero maternal stem cell transplantation for Fanconi anemia (FA). I am a physician by training. I grew up in the Bay Area and now live in Marin County. I work in clinical genomics as the CEO of [jscreen.org](https://www.jscreen.org), the only national non-profit providing education, access, and high-touch support for preventive genetics including carrier and hereditary cancer testing. But today I write in a personal capacity, and as a parent.

In December 2019, our daughter Havi was diagnosed with Tay-Sachs disease. She was just over a year old. Over the following year, my wife and I watched her lose, one by one, every skill she had gained: feeding herself, then eating from a spoon, then sleeping through the night without pain. There was no treatment to try and no trial to enroll in. As a physician I was used to having a next step, and there was none. Our medical system could name what was happening to her, but it could not slow it. Havi died in January 2021. She was two.

I tell you this because the CIRM Team's memo describes this application in terms I recognize. It is an ultra-rare disease, with roughly four affected births a year in California. The reachable population is small, and further development would remain after the award. Every one of those statements was also true of Tay-Sachs. Those are the reasons no one built a therapy that could have helped my daughter. They are not reasons for a public agency to step back. They are the reason a public agency exists.

My work since Havi's death has focused on carrier screening, and that experience speaks directly to the memo's central concern: whether eligible pregnancies can be identified in time. They can. FA genes are included on most expanded carrier screening panels, and professional guidance increasingly supports offering broad screening to everyone who is pregnant or planning a pregnancy. Carrier couples can obtain a diagnosis early enough in pregnancy to consider in utero treatment. The most reachable families of all are those who already have a child with FA. Each of their pregnancies carries a one-in-four risk, and they are deeply motivated to find another path. Screening uptake is rising and costs are falling, so the eligible population will grow over the life of this award.

But screening alone is not enough, and I say that as someone who has devoted himself to it. Today, a couple who learns their pregnancy is affected faces only hard choices. What this trial offers is something families like mine never had: a way to treat a child before the disease takes hold. For FA, that matters especially. Conventional transplant requires conditioning that damages DNA, in children whose disease is defined by an inability to repair it. This approach avoids conditioning, needs no matched donor, and works across FA genotypes. If it succeeds, it will also point the way for other conditions that can be diagnosed before birth.

This application earned the top score in the round, with unanimous Grants Working Group support. Its cost is modest relative to the clinical budget remaining this cycle. If the Committee shares staff concerns about enrollment, I ask that it fund the award with milestone conditions, such as an enrollment benchmark at 18 to 24 months, rather than decline it outright.

Parents of children with ultra-rare diseases learn quickly that the market was not built for them. Proposition 14 was meant to be different. I respectfully ask you to fund CLIN2-20316.

Sincerely,  
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