



541.687.4658 | info@fanconi.org

360 E 10th Ave., Suite 201 Eugene, OR. 97401

September 24, 2026

To: CIRM ICOC Committee

Re: Strong Support for Funding CLIN2-20316: In Utero Hematopoietic Stem Cell Transplantation for Fanconi Anemia

Dear Members of the CIRM ICOC Committee:

It is with deep compassion and urgency that we the undersigned members of the greater community of Fanconi anemia (FA) patients, advocates, caregivers, donors, and friends, plead for funding **CLIN2-20316: In Utero Stem Cell Transplantation to Prevent Bone Marrow Failure and Cancer in Fanconi Anemia**, a clinically ready and potentially transformative approach to treating the blood and immune defects in our children with Fanconi anemia (FA) – an approach that could prevent bone marrow failure and cancer in these fragile FA patients. The proposed program addresses a profound unmet medical need within our community with a scientifically compelling, operationally feasible, and equity-centered preventative potential treatment, and we are determined to assist in enabling this potentially ideal new treatment option to become available for our community.

We understand that with unanimous support of the CIRM Grants Working Group reviewers, as a top scoring CLIN2 application, there is no question as to the scientific merit of this proposal. It offers an entirely new framework to the current challenging treatment options for FA by enabling immediate prevention rather than late-stage salvage in the setting of advanced disease. This application aims to advance in utero hematopoietic stem cell transplantation (IUHSCT), an innovative new treatment for fetuses diagnosed prenatally with FA using a stem cell product made from the mother's bone marrow. By taking advantage of a natural time window in which the fetal immune system is tolerant to mom's cells, the approach aims to stabilize the blood and immune system without the chemotherapy/irradiation conditioning or immunosuppression that are required after birth and particularly toxic to FA patients. This treatment aims to fundamentally change FA disease, by eliminating years of anxiety waiting for bone marrow failure, the challenging current transplant course and is expected to reduce the risk of deadly cancers that plague FA patients, functioning as a gateway to cancer prevention.

We are impressed by the outstanding, multidisciplinary team leading this proposal and their deep expertise in FA, fetal intervention, hematopoietic transplantation, GMP manufacturing, regulatory affairs, immune monitoring, clinical operations, data management, and long-term patient follow-up. Having supported previous research by both Stanford and UCSF investigators paving the way for better treatment options including this new approach, we are in steadfast support of this proposal. In fact, the Fanconi Cancer Foundation has already committed financial



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support for the preclinical work leading to the development of this trial and intends to provide funding support for the trial, contingent upon final review and approval by our Board of Directors on October 2, 2027. We believe that the experts leading this study have the experience and expertise needed to be successful in advancing prenatal transplant for FA, however we do not have the financial means to support the full study and implore CIRM to help fill this critical funding void to enable this clinical trial.

FA is an ultra-rare disease, though there are thousands of patients challenged by FA that reside in California, across the United States and worldwide. Given Dr. Czechowicz's extensive presence and involvement with the FA community, including in other successful FA clinical trials, we can attest to the strong relationships and connections that Dr. Czechowicz has fostered in our community. Therefore, we do not see any challenges with spreading awareness both locally and globally about this trial, its potential impact on families, and how to participate. With our collective efforts, we anticipate enrollment success and have already been working with the team to generate educational materials and patient support for the study through our dedicated FA advocates. Given regulatory clearance to begin the trial, Dr. Czechowicz and team were hopeful to announce the trial's opening during Fanconi Cancer Foundation's Annual Scientific Symposium next week. However, we are nervous to do this without the commitment of funding as we do not want to give families false hope. We anticipate a cascade of support not only from symposium attendees but also from FA patient advocates from all over the world which could aid in study awareness and subsequent recruitment, and we have already introduced several families with a prenatal diagnosis to Dr. Czechowicz and her team. As members of the FA community have been actively engaged in providing feedback for this trial – from completing surveys about their experience with FA to meeting monthly with the study team – we are confident that this trial has been built with utmost attentiveness to a potential FA trial participant's needs.

This equity-centered proposal also addresses a large unmet need within California's diverse population, including Hispanic/Latino families who comprise a substantial portion of FA patients seen at Stanford and UCSF which is in part due to founder mutations in these communities. These patients may face disproportionate barriers related to insurance, language, geography, and access to specialized surveillance and transplantation services. A preventive, maternal-donor approach has the potential to reduce—not deepen—these longstanding inequities which would be further minimized by the potential for FA prenatal screening that could be standardized because of this work. Additionally, the potential health-system impact is substantial. If successful, a single prenatal infusion could avert or delay bone marrow failure, reduce dependence on transfusions and postnatal transplantation, lower the risk of transplant-related complications and secondary cancers, and spare families years of intensive surveillance, hospitalization, uncertainty, and caregiver burden. More importantly, it could give



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children with FA to have a normal childhood without the stress, anxiety and fatigue that comes with the expectation of bone marrow failure and transplantation. Beyond treatment for FA, this work may also help other patients and rare genetic disease communities and catalyze improved prenatal and family-based genetic screening and treatment, allowing care to shift from late-stage treatment toward prevention.

We understand the urgency better than anyone else in helping families who are confronting a prenatal diagnosis of FA and who must make extraordinarily difficult decisions in the setting of limited therapeutic options and the prospect of lifelong medical complications. This proposal gives those families – our families – a scientifically grounded, carefully regulated, and ethically reviewed opportunity to pursue a preventive treatment strategy. Given Dr. Czechowicz’s endless hard work and relentless perseverance, we have a deep belief that she and her team will find a way to develop this stem cell therapy into an accessible treatment for our FA community. However, without CIRM support, there is no realistic pathway to generate the clinical evidence needed to advance this innovative approach.

We therefore respectfully urge CIRM to provide funding for CLIN2-20316: In Utero Stem Cell Transplantation to Prevent Bone Marrow Failure and Cancer in Fanconi Anemia.

Sincerely,

The FA Community:

A handwritten signature in black ink that reads "Isis Sroka".

Isis Sroka, PhD
CEO Fanconi Cancer Foundation

“As a father of two daughters with FA, I cannot urge CIRM enough to consider funding this research, which could mean a world without cancer and bone marrow failure for those almost certain to become afflicted. The direct beneficial impact cannot be overstated of what this trial seems to be able to accomplish.”

A handwritten signature in black ink, appearing to be "Bill Katris".

Bill Katris
Fanconi Cancer Foundation Board of Directors
FA Parent, Illinois



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"After watching both of my children battle Fanconi anemia and undergo bone marrow transplants, I support this research because no family should have to endure that journey if a safer, preventive option can become a reality."

A handwritten signature in black ink, appearing to read "Erica Parra Williams".

Erica Parra Williams
FA Parent, New Jersey

"As one of many California families living with this condition, we are passionate about the work of Dr. Czechowicz and this potentially curative treatment for this profoundly challenging condition."

A handwritten signature in black ink, appearing to read "Zach Gratz-Lazarus". The word "Signature:" is printed to the left of the signature.

Zach Gratz-Lazarus
FA Parent, California

"My son went through a bone marrow transplant at age nine, and now we live with the worry of cancer every day. This research could mean the next generation of FA families never has to face this."

A handwritten signature in black ink, appearing to read "Sarah Borden".

Sarah Borden
FA Parent, Wisconsin

"This research gives the FA community something we all hold on to: hope for a better future and more options for the next generation."

A handwritten signature in black ink, appearing to read "Christina Faur".

Christina Faur
FA Parent, United Kingdom



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“Having lived 30 years with the threat of a BMT hanging over my head and finally going through with it due to MDS/bone marrow failure 6 years ago, I can only imagine how wonderful it would be if transplant could be treated before birth. On behalf of future FA patients and families, I strongly support this initiative.”

Egil Dennerline

Egil Dennerline
FA Adult, Denmark

“As an FA advocate and parent, I strongly believe that families need innovative treatment options that can prevent bone marrow failure and improve outcomes for children with FA.”

A handwritten signature in grey ink, appearing to read "S. Sanyanga".

Sylvia Sanyanga
FA Parent, Zimbabwe

“My daughter has endured 27 bone marrow biopsies and she’s only 18 years old. I wish I could go back in time and participate in this innovative therapy and prevent her from going through so many biopsies. Please support this therapy so future children with FA will avoid the pain and suffering that comes with so many bone marrow biopsies.”

A handwritten signature in grey ink, appearing to read "Tricia Mitchell".

Tricia Mitchell
FA Parent, Maryland

“This type of cutting edge clinical trial is exactly the hope FA families need that better treatments are coming, and that maybe someday a cure is possible.”

A handwritten signature in grey ink, appearing to read "Kelly McKenna".

Kelly McKenna
FA Parent, Ohio



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“Cada investigación sobre esta condición, cada tratamiento se traduce en oportunidades para nuestra comunidad. Salva vidas, cultiva esperanza. Tiene una fuerza transformadora.”

English Translation:

"Every research study on this condition and every treatment creates new opportunities for our community. It saves lives and cultivates hope. It has a transformative power."

A handwritten signature in black ink that reads "Karen Gonzalez". The script is fluid and cursive.

Karen Gonzalez
FA Parent, Uruguay

“As an adult living with Fanconi anemia who has undergone bone marrow transplantation and experienced many of the lifelong complications of FA, I believe research that could intervene before bone marrow failure begins represents extraordinary hope for future generations.”

A handwritten signature in black ink that reads "Dexter Sherrell". The script is cursive and elegant.

Dexter Sherrell
FA Adult, Georgia

“As someone who has lived with Fanconi anemia since childhood, I hope this research can give future babies with FA a different beginning than the one many of us had.”

A handwritten signature in black ink that reads "Maria Isabel Rodriguez". The script is cursive and somewhat stylized.

Maria Isabel Rodriguez
FA Adult, Colombia

A handwritten signature in black ink that reads "Fatma Issak". The script is cursive and bold.

Fatma Issak
FA Adult, New York



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A handwritten signature in black ink that reads "Mary Eileen Cleary".

Mary Eileen Cleary
FA Parent, Michigan

A handwritten signature in black ink that reads "Mahazareen Dubash".

Mahazareen Dubash
FA Parent, Canada

A handwritten signature in blue ink that reads "Alejandra Tabar".

Ana Alejandra Tabar Concha
FA Adult, Dominican Republic

A handwritten signature in black ink that reads "Fiona Chong".

Fiona Chong
FA Adult, Australia