Stanford MEDICINE
Children's Health

Bass Center for Childhood Cancer and Blood Diseases

Our Approach to Treating Fanconi Anemia (FA)

Fanconi anemia is a rare, genetic disease that causes every cell in the body to be vulnerable to DNA damage, leading to the death of vital cells or contributing to their transformation into cancers. Through innovative research, Stanford Children's Health has developed groundbreaking new treatments for FA that help children and adults survive and thrive.

Why Stanford Children's Health for Fanconi anemia

- Our **team of care providers** are among the top doctors in the nation and world for FA; we have handpicked them for their complementary expertise and innovative approaches to treating FA. Our team is truly committed to optimal FA care and includes hematologists, stem transplant specialists, ENTs, gynecologists, dermatologists, genetic counselors, social workers, child life specialists and other subspecialty providers. Many on our team have decades of experience caring for FA patients, and we closely follow the evolving standards and advances in the FA field.
- We are a center of excellence for Fanconi anemia care and research and the leading FA treatment center in the West, offering multidisciplinary care and treating children and adults of all ages from California, the nation, and the world.
- Our FA program is housed in our **Bass Center for Childhood Cancer and Blood Diseases**, which is nationally known for its **innovative research in cancer and cell and gene therapy**. Our **Center for Definitive and Curative Medicine**, accelerates the process of translating research insights into new treatments to bring life-changing therapies to patients, including people with FA.

Unique research-driven treatments

While we provide exceptional standard of care **treatments for FA**, including comprehensive disease monitoring, growth factor therapies, blood transfusions, and stem cell transplants, we are especially proud to also offer leading-edge and novel investigational treatments for FA. These include:

- **Personalized diagnostics and decision-making.** We are one of the few programs in the nation to conduct thorough diagnostic testing to understand the complete genetic cause of FA in your child. We are one of a few programs in the nation to conduct diagnostic testing to understand the complete genetic cause of FA in your child, and we additionally encourage detailed testing to understand the full extent of disease in your child's blood and bone marrow. This information can then be used to tailor our treatments specific to your child, homing in on exactly what care your child needs at each step throughout his or her medical journey.
- **Novel approaches to stem cell transplants.** Stem cell transplants (sometimes called bone marrow transplants) are the only recognized cure for the blood problems associated with FA. We take a unique approach to transplants, including:

  — **Exceptional planning and monitoring.** Our program has decades of expertise in transplanting patients with FA. We carefully tailor treatment regimens for each patient, considering their disease state and donor options. We pay close attention to detail before, during, and after the transplant to ensure excellent outcomes, and our center has a wide variety of innovative treatments and outstanding subspeciality teams available if issues such as graft-versus-host disease (GvHD) or infections unexpectedly arise.

  — **Alternatives for conditioning.** Conditioning is the essential part of the patient preparation for a **stem cell transplant**. Because FA patients are particularly sensitive to chemotherapy and radiation, which are traditionally used in transplants, we tailor our conditioning to reduce the use of these agents. Using a combination of low-dose chemotherapy and low-dose radiation while shielding vital organs from the radiation, we have exceptional transplant outcomes. We are also developing methods to **eliminate alkylating chemotherapy and radiation**, and recently **published the successful results** of the first clinical trial with reduced-intensity antibody-based conditioning. This trial has had promising results, and we are making additional adjustments to help patients further in the future, including through reducing use of the antibody.

  — **Graft manipulation.** One way to increase the likelihood of stem cells growing quickly after transplant and decrease the risk of side effects like GvHD is to manipulate the graft to increase the number of stem cells and eliminate undesirable immune cells that cause GvHD. Our center has unique expertise in manipulating grafts, using a technique called **TCR alpha/beta T-cell/CD19 B-cell depleted stem cell transplant**. For patients without matched sibling donors, we have adopted this graft manipulation as our hospital's standard approach to care for patients with certain genetic conditions, including FA. By using a child's parents or siblings for this approach, we also have full control of the collection process to ensure an optimal stem cell graft and remove the need for graft shipment to further minimize risks.

- **Prenatal counseling and treatment.** As FA is a genetic disease, it most often results from mutations that are passed down by parents. We offer genetic counseling, family planning, and in vitro fertilization to assist in preventing FA from being passed on. Additionally, during pregnancy, our multidisciplinary team can assist with prenatal testing and counseling if there is a concern for FA in the developing fetus. For families with a prenatal FA diagnosis, we have been developing prenatal treatment approaches using maternal bone marrow cells that can be transplanted in utero into the fetus without any conditioning. We have a **clinical trial in development to enable preventive treatment of FA before birth** using this approach.
- **Long-term follow up.** Although our stem cell transplant treatments have exceptional outcomes, they still place patients at risk of short-term and long-term complications. Additionally, even without transplant, FA patients can have various health issues, so we follow all of our patients closely. Initially post-transplant, patients stay in the hospital and then in the local vicinity, and once patients have stable recovery of their immune system, they return home while maintaining close communication with our team. We can use telehealth services to provide care at a distance, work closely with local care providers for laboratory work, exams and treatments, and encourage patients to return annually for further follow up. In this way, patients living hundreds and even thousands of miles away from Stanford can continue to get excellent care after their transplants. Our team primarily cares for children; however, we also provide care for adults with FA by close collaboration between the pediatric and adult transplant teams.
- **Innovative cancer treatments.** Fanconi anemia puts patients at a higher risk for cancers, especially **leukemia**, a blood cancer. While our goal is to prevent such cancers in FA patients, we also offer the very latest **research-driven cancer treatments** for patients who do unfortunately develop various cancers, and we closely monitor our patients to be able to treat these as early as possible to improve their outcomes.

Alternative treatments in development

Our physicians and researchers are working on many new therapies that, while not currently available, will hopefully be an option for patients in the near future.

- **New oral medicines.** We are studying the investigational use of alternative oral medicines with the hope of improving the performance of the blood cells and preventing cancer. For example, our group has assisted in developing a drug called an **ALDH activator**, specifically FP-045, in FA patients to test if it can restore stem cells to normal function and prevent damage.
- **Gene therapy.** For Fanconi patients with subtype A (FA-A), we previously offered an investigative **gene therapy treatment** that is intended to reverse the root cause of the blood disorders associated with FA by placing an intact normal copy of the FANCA gene (the gene that causes FA in a majority of patients) inside the blood stem cells of patients. The goal of this gene therapy is to enable the corrected blood stem cells to grow normally, thereby avoiding or delaying bone marrow failure in children. We obtained promising results in our recent clinical trials showing the safety of this treatment as well as early efficacy. While this treatment is not currently available, we hope to be able to offer it or other gene therapy treatments to more patients in the future.

For More Information

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M054594 | 03/2026