



Now Enrolling Patients

Antibody-Based Conditioning with TCRab T-cell/CD19 B-cell Depleted
Allogeneic Transplantation for Fanconi Anemia Patients with Cytopenias

What is Fanconi Anemia (FA)?

FA is a rare genetic disorder affecting DNA repair that is caused by diverse genetic defects. Approximately 90% of patients develop bone marrow failure (very low blood counts), and many also develop cancers of the blood or other tissues.

How does this treatment work?

Blood stem cells are collected from healthy donors and isolated from problematic T-cells. These healthy stem cells are then given to the patient by intravenous infusion after treatment of the patient with preparative medications containing standard immune suppression and a non-genotoxic anti-CD117 antibody (JSP191). Unlike other FA transplant treatments, no irradiation or busulfan is used.



Who is eligible to participate in this clinical trial?

All Fanconi Anemia patients of all subtypes ages 2+, who have developed cytopenias and do not have an HLA-identical matched sibling donor for bone marrow transplant, and are not on other experimental therapies at the time or have active cancers or concerns for high-risk bone marrow disease.

What does participation in this clinical trial involve?

Before you begin the study, you will have to have several tests to determine whether you are eligible to join the trial. These tests can take up to 3 months to complete. After the doctors conducting the study confirm that you are eligible for the trial, participating in the study will involve:

- **Conditioning:** To prevent rejection of the donor cells, patients will be treated with standard immune suppression and an antibody-drug, JSP191, directed against stem cells in place of genotoxic irradiation or busulfan treatment.
- **Infusion of Donor Stem Cells:** Stem cells will be collected from an eligible donor and then purified to removed T-cells that increase the likelihood of graft vs host disease. These will be infused through a special catheter.
- **Follow-up after Transplant:** Patients will initially be monitored in the hospital and then will need to return for follow-up visits, including blood and bone marrow tests, over the next 2 years. In addition, patients will have long-term follow-up approximately 1-2 times per year for another 8 years.

How much will it cost to participate in the trial?

Financial support, including travel arrangements and housing accommodations for patients and a family member, both for the treatment and follow-up visits, will be coordinated with insurance.

If you would like to learn more about this clinical trial, call or email:

Stanford University (Palo Alto, CA, USA)
Bone Marrow Failure Program
650-497-8953
bmf@stanfordchildrens.org

For questions about participants' rights, contact 1-866-680-2906