Global Gene Therapy
Clinical Trial for Fanconi Anemia Type A (FA-A)

NOW ENROLLING PATIENTS

If you would like to learn more about the gene therapy clinical trial, call or email:

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

Or visit:
https://clinicaltrials.gov/ct2/show/NCT03814408

What is Fanconi anemia (FA)?
FA is a rare genetic disorder affecting DNA repair. Approximately two-thirds are caused by genetic defects in the FANCA gene, which causes the FA subtype known as FA Complementation Group A (FA-A). FA patients may develop bone marrow failure (very low blood counts) and cancers of the blood or other cancers.

How does this gene therapy work?
Blood stem cells collected from the FA-A patient are genetically modified to introduce an intact copy of the FANCA gene using a virus that has been changed in the laboratory so that it cannot grow or spread to cause an infection. The genetically modified cells are then returned back into the patient.

Who is eligible to participate in the gene therapy clinical trial?
FA-A patients ages 1 through 12, who have not developed severe bone marrow failure, do not have an HLA-matched sibling donor for bone marrow transplant, and are not on other experimental therapies.

What does participation in the gene therapy 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 1 month to complete.

After the doctors conducting the study confirm that you are eligible for the trial, participating in the study will involve:

- **Stem Cell Collection**: Stem cells have the potential to make the different blood cells in your body. You will receive medications that make stem cells in your bone marrow enter your blood where they will be collected from your vein (most likely using a catheter) during a procedure called apheresis.

- **Infusion of Genetically Modified Cells**: Your stem cells will be genetically changed in a laboratory to introduce the intact copy of the FANCA gene. You will then receive an infusion of the gene-modified cells through the catheter. There is no conditioning chemotherapy before the infusion.

- **Follow-up after Gene Therapy**: Patients will need to return for follow-up visits, including blood and bone marrow tests, over the next 3 years. In addition, patients will have long-term follow-up with their home physician approximately 1-2 times per year for another 12 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 provided.