Gene Therapy Clinical Trial for Fanconi Anemia Type A (FA-A)
NOW ENROLLING PATIENTS

What is Fanconi anemia (FA)?
FA is a rare genetic disorder affecting DNA repair. Approximately 2/3 of cases are caused by mutations in the FANCA gene, resulting in FA Complementation Group A. Most patients develop bone marrow failure during the first decade of life; about a quarter develop leukemia or myelodysplastic syndrome (MDS); and a third develop squamous cell carcinoma or other solid tumors.

What is the purpose of this gene therapy clinical trial?
This study is an open-label pediatric trial to assess the use of autologous hematopoietic stem and progenitor cells (HSPCs) transduced ex vivo with a lentiviral vector (LV) encoding for the FANCA gene to treat FA-A, to prevent hematologic progression to severe bone marrow failure requiring allogeneic hematopoietic cell transplantation.

Who is eligible to participate in the gene therapy clinical trial?
FANCA patients ages 1 through 12 with mild cytopenias, who have not developed severe bone marrow failure or dysplasia, do not have an HLA-identical sibling donor, and are not on other experimental therapies. The trial is expected to enroll 12 patients globally.

Where will the gene therapy clinical trial be conducted?
Two clinical centers, one in Europe and one in the US will be enrolling FA-A patients globally following very similar protocols. In the US, the trial will be conducted at Stanford University in Palo Alto, California, with Dr. Sandeep Soni and Dr. Agnieszka Czechowicz as the clinical investigators, and Dr. Maria Grazia Roncarolo as the Chief of the Division of Pediatric Stem Cell Transplantation and Regenerative Medicine and the Director for the Center for Definitive and Curative Medicine.

What does participation in the trial entail?
- Hematopoietic stem and progenitor cells (HSPCs) will be mobilized and collected from the peripheral blood of FA-A patients, which will take approximately 6 days. Mobilization is performed via several days of G-CSF and plerixafor (AMD-3100) injections. Collection is performed via apheresis, requiring a central catheter.
- Following HSPC collection, ex vivo transduction with the LV encoding for FANCA occurs over 2-3 days. The patient will then receive a single intravenous infusion of gene-corrected cells over 1 hour or less with no prior conditioning chemotherapy.
- Patients will be hospitalized from mobilization of HSPCs to infusion of the gene therapy. They will be discharged from the hospital approximately 2 days after the gene therapy infusion.
- Follow-up at the clinical center is expected to span over the next 3 years, with more frequent visits in the weeks/months immediately following treatment.
- Long-term follow-up with the patient’s home physician is anticipated to occur approximately 1-2 times per year for an additional 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.
IF YOU WOULD LIKE TO LEARN MORE ABOUT THE GENE THERAPY CLINICAL TRIAL CONTACT:

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For more information, visit:
[https://clinicaltrials.gov/ct2/show/NCT03814408]