
Gene Transfer for Artemis-Deficient Severe Combined Immunodeficiency Using a Lentiviral Vector to Transduce Autologous CD34 Hematopoietic Stem Cells

Grant Award Details

Gene Transfer for Artemis-Deficient Severe Combined Immunodeficiency Using a Lentiviral Vector to Transduce Autologous CD34 Hematopoietic Stem Cells

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-10830

Investigator:

Name:	Morton Cowan
Institution:	University of California, San Francisco
Type:	PI

Disease Focus: Blood Disorders, Severe Combined Immunodeficiency, Artemis deficient (ART-SCID)

Human Stem Cell Use: Adult Stem Cell

Award Value: \$12,000,000

Status: Active

Grant Application Details

Application Title: Gene Transfer for Artemis-Deficient Severe Combined Immunodeficiency Using a Lentiviral Vector to Transduce Autologous CD34 Hematopoietic Stem Cells

Public Abstract:**Therapeutic Candidate or Device**

Bone marrow stem cells that have been treated by inserting a normal Artemis gene into the DNA using a modified virus called a lentivirus.

Indication

Children with severe combined immunodeficiency or "bubble baby disease" due to a defective gene that makes a protein called Artemis

Therapeutic Mechanism

Stem cells from the bone marrow normally generate key components of the immune system including T and B cells. Children with Artemis deficiency do not make T or B cells. Gene therapy for these patients will involve correcting the patient's own bone marrow stem cells by inserting a normal Artemis gene into the DNA so that normal T and B cells can be produced, thus completely correcting this immunodeficiency.

Unmet Medical Need

Artemis deficiency is the most difficult form of SCID to treat with a bone marrow transplant from a healthy donor; serious complications are much more likely than for other forms of SCID. Using gene-corrected stem cells from the patient should eliminate these issues while restoring normal immunity.

Project Objective

Complete a phase 1 trial of toxicity/feasibility.

Major Proposed Activities

- Complete a trial to assess the clinical safety of gene-corrected patient's stem cells in babies and older children with Artemis deficient SCID
- Determine the feasibility of restoring normal T and B cell immunity with gene-corrected patient's stem cells
- Use special research assays to characterize completeness of T, B and NK cells restoration

Statement of Benefit to California:

California was one of the initial states to implement newborn screening for severe combined immunodeficiency (SCID) and has the largest experience with SCID due to its population and birth rate. Families with babies diagnosed with Artemis-deficient SCID will potentially benefit from this novel therapy. Results from this study will advance our knowledge of the risks and benefits of this type of gene therapy that is likely to be applicable to other disorders involving bone marrow stem cells.

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