Lentiviral Gene Therapy for Infants with X-linked Severe Combined Immunodeficiency using Autologous Bone Marrow Stem Cells and Busulfan Conditioning

Grant Award Details

Lentiviral Gene Therapy for Infants with X-linked Severe Combined Immunodeficiency using Autologous Bone Marrow Stem Cells and Busulfan Conditioning

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-09504

Project Objective: Complete a Phase 1 clinical trial for infants with X-linked Severe Combined Immunodeficiency with lentivirus transduced autologous bone marrow stem cells and Busulphan conditioning.

Investigator:

Name:	Stephen Gottschalk
Institution:	St. Jude Children's Research Hospital
Type:	PI

Disease Focus: Blood Disorders, Immune Disease, Severe Combined Immunodeficiency, X-linked (X-SCID)

Human Stem Cell Use: Adult Stem Cell

Award Value: $11,924,780

Status: Active

Grant Application Details

Application Title: Lentiviral Gene Therapy for Infants with X-linked Severe Combined Immunodeficiency using Autologous Bone Marrow Stem Cells and Busulfan Conditioning

Public Abstract:

Therapeutic Candidate or Device

Bone marrow stem cells will be transduced with a lentiviral vector to deliver a normal copy of the gamma-chain gene to treat X-linked SCID.

Indication

x-linked severe combined immunodeficiency, a severe pediatric disorder in which children have multiple defects in their immune system.

Therapeutic Mechanism

The gene therapy will correct the patient's own bone marrow stem cells. Transplantation with these corrected cells will lead to restored production of T, B, and NK lymphocytes resulting in correction of the underlying immunoeficiency disorder. Correction of the immune cells will allow the patients to fight infections that are life-threatening without curative therapy. The patients will be spared life-long supplementation with expensive preparations of human antibody with B cell correction.

Unmet Medical Need

XSCID is a catastrophic disease of childhood. Children with matched sibling donors do well with bone marrow transplant, but most cases lack a matched sibling donor. We address this unmet need by using the patient's own bone marrow stem cells for transplantation after lentiviral transduction.

Project Objective

Phase I/II trial completed.

Major Proposed Activities

- Open LVXSCID-ND trial at UCSF.
- Enroll at least 6 patients from California at the UCSF performance site.
- Analyze immune reconstitution and safety in XSCID gene therapy patients.

Statement of Benefit to California:

UCSF is a major referral site for XSCID patients in California. If this gene therapy works as intended, these children will be provided curative therapy without any risk of graft-versus-host disease. Under current conditions, treatment for XSCID patients that lack matched sibling donors is very expensive, and for those that lack health insurance, a major expense for their state of residence. Lastly, this may provide commercialization opportunities for biotech companies in California.

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