Stem Cell Gene Therapy for HIV Mediated by Lentivector Transduced, Pre-selected CD34+ Cells in AIDS lymphoma patients

Grant Award Details

Stem Cell Gene Therapy for HIV Mediated by Lentivector Transduced, Pre-selected CD34+ Cells in AIDS lymphoma patients

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-08289

Project Objective: Complete a Phase I clinical trial for AIDS Lymphoma with Lentivirus transduced pre-selected autologous CD34+ hematopoietic stem cells.

Investigator:

Name:	Mehrdad Abedi
Institution:	University of California, Davis
Type:	PI

Disease Focus: Blood Cancer, Cancer, HIV-related Lymphoma, HIV/AIDS, Infectious Disease

Human Stem Cell Use: Adult Stem Cell

Award Value: $8,414,265

Status: Active

Progress Reports

Reporting Period: Operational Milestone #1

View Report

Grant Application Details

Application Title: Stem Cell Gene Therapy for HIV Mediated by Lentivector Transduced, Pre-selected CD34+ Cells in AIDS lymphoma patients

Public Abstract:

Therapeutic Candidate or Device

HSC gene modified by a lentiviral vector which encodes a triple combination of HIV-resistance genes and a pre-selective marker.

Indication

HIV in AIDS-lymphoma patients.

Therapeutic Mechanism

Bone marrow transplants are standard of care for AIDS-lymphoma patients providing a cure for the patient's lymphoma. A purified population of triple combination anti-HIV lentiviral vector transduced CD34+ HSCs will safely engraft, divide, and differentiate in vivo into a pool of mature myeloid and lymphoid cells. This newly generated HIV-resistant immune system will be capable of blocking further HIV infection.

Unmet Medical Need

HIV continues to be a public health problem worldwide with no effective vaccine or cure available. Despite anti-retroviral therapy prolonging lives of patients, it is not curative. HIV stem cell gene therapy provides the potential to replace a patients immune system with one resistant to HIV.

Project Objective

To complete a Phase I clinical trial.

Major Proposed Activities

- Evaluate the coorelatives of transplanted cells including DNA, immune, and virological monitoring.
- To conduct a Phase I study of safety, feasability, and efficacy of our product in AIDS-lymphoma patients.
- To manufacture and quality control GMP grade anti-HIV lentivector and clinical grade HIV-resistant HSC.

Statement of Benefit to California:

In California, the number of HIV infected individuals continues to increase. As anti-retroviral drugs are not curative, these individuals still have to deal with the emotional, financial, and medical consequences. Our HIV stem cell gene therapy approach comprises the transplantation of a purified population of HIV-resistant blood forming stem cells which would generate an HIV-resistant immune system in a patient's body. This would be significantly compelling to the state of California.
