Phase 1/2 study for autologous human CD34+ hematopoietic stem cells ex vivo transduced with pCCL-CTNS lentiviral vector for treatment of Cystinosis.

Grant Award Details

Phase 1/2 study for autologous human CD34+ hematopoietic stem cells ex vivo transduced with pCCL-CTNS lentiviral vector for treatment of Cystinosis.

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-11478

Project Objective: Complete a Phase 1/2 study using autologous hematopoietic stem cells modified with lentivirally-delivered CTNS1 gene for treatment of cystinosis.

Investigator:

Name:	Stephanie Cherqui
Institution:	University of California, San Diego
Type:	PI

Disease Focus: Cystinosis, Kidney Disease, Kidney Failure, Metabolic Disorders

Human Stem Cell Use: Adult Stem Cell

Award Value: $11,999,944

Status: Active

Grant Application Details

Application Title: Phase 1/2 study for autologous human CD34+ hematopoietic stem cells ex vivo transduced with pCCL-CTNS lentiviral vector for treatment of Cystinosis.

Public Abstract:

Therapeutic Candidate or Device

Autologous Human CD34+ HSC from Mobilized PBSC of Patients with Cystinosis Modified by Ex Vivo Transduction using the pCCL-CTNS Lentiviral Vector

Indication

Cystinosis - An autosomal metabolic disease that belongs to the family of the lysosomal storage disorders. Gene involved is CTNS (encodes cystinosin).

Therapeutic Mechanism

The proposed therapy intervention is intended to impact the target indication of Cystinosis via autologous tranplantation of CD34+ HSC-mediated transfer of a functional cDNA using pCCL-CTNS lentivirus vector. The gene-corrected HSC progeny will differentiate into macrophages in injured tissues and transfer cystinosin-bearing lysosomes via Tunneling Nanotubes (TNTs) to disease cells. This transfer of functional cystinosin to endogenous tissue cells leads to long-term tissue preservation.

Unmet Medical Need

The only treatment available for cystinosis is a lifetime oral cysteamine, with severe side effects and compliance challenges, that only delays the disease complications. This approach may represent a one-time life-long therapy that may prevent kidney transplantation and quality of life of patients.

Project Objective

Phase 1/2 trial completed

Major Proposed Activities

- Clinical:
  - Screening and Enrollment
  - Product Administration
  - Clinical Monitoring/Safety Assessments by DSMB (IQVIA)
  - 24-month Patient Follow-Up
- Manufacture clinical product for the proposed trial:
  - Mobilization and Leukapheresis
  - CD34+ Isolation & Transduction
  - Release Testing & Infusion

Statement of Benefit to California:

California has approximately 20 cystinosis patients, and their families, who could directly benefit from this treatment. Financial burden on MediCal is expected to by reducing or eliminating the costs of Cysteamine and the treatment cost of secondary conditions such as hypothryodism, polyurea, etc (cost range per patient ~$300,000-600,0000/year). Moreover, at least 80% of the funds spent will be within the state of California.

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