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**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**

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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:** A Phase 1/2 study to determine the safety and efficacy of transplantation with autologous human CD34+ hematopoietic stem cells (HSC) from mobilized peripheral blood stem cells (PBSC) of patients with cystinosis modified by ex vivo transduction using the pCCL-CTNS lentiviral vector

**Investigator:**

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<b>Institution:</b>	University of California, San Diego
<b>Type:</b>	PI

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**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**

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**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 transplantation 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 be reduced or eliminated by reducing or eliminating the costs of Cysteamine and the treatment cost of secondary conditions such as hypothyroidism, polyurea, etc (cost range per patient ~\$300,000-600,000/year). Moreover, at least 80% of the funds spent will be within the state of California.

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**Source URL:** <https://www.cirm.ca.gov/our-progress/awards/phase-12-study-autologous-human-cd34-hematopoietic-stem-cells-ex-vivo-transduced>