A Phase I Clinical Trial for a Lentiviral Gene Therapy Targeting the TCIRG1 Gene for Infantile Malignant Osteopetrosis (IMO)

Grant Award Details

A Phase I Clinical Trial for a Lentiviral Gene Therapy Targeting the TCIRG1 Gene for Infantile Malignant Osteopetrosis (IMO)

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-12095

Project Objective: Conduct a Phase 1 trial to assess the safety and preliminary efficacy of transplanted, autologous, lentivirally transduced, TCIRG1-expressing hematopoeitic stem cells in children with Infantile Malignant Osteopetrosis.

Investigator:

Name:	Gayatri Rao
Institution:	Rocket Pharmaceuticals, Inc.
Type:	PI

Disease Focus: Bone or Cartilage Disease

Human Stem Cell Use: Adult Stem Cell

Award Value: $3,728,485

Status: Active

Grant Application Details

Application Title: A Phase I Clinical Trial for a Lentiviral Gene Therapy Targeting the TCIRG1 Gene for Infantile Malignant Osteopetrosis (IMO)

Public Abstract:

Therapeutic Candidate or Device

The therapeutic candidate is an ex-vivo autologous gene therapy approach for Infantile Malignant Osteopetrosis (IMO).

Indication

The target clinical indication is Infantile Malignant Osteopetrosis (IMO), a pediatric, autosomal recessive rare disease.

Therapeutic Mechanism

The therapeutic mechanism is based on an ex-vivo, lentiviral-based, patient-specific approach by genetically engineering the patient's own CD34+ selected cells with the corrected genetic sequence of TCIRG1 gene and subsequently infusing the corrected cells to deliver a potential cure.

Unmet Medical Need

Children with severe IMO face morbid conditions such as hematologic and/or neurologic deficits that worsen over time . There is a high chance of death in the first decade of life without successful allogeneic HSCT. This gene therapy will treat the underlying TCIRG1 mutation in IMO patients.

Project Objective

Phase 1 trial completed

Major Proposed Activities

- Patient recruitment, screening, and support (by various CROs) on their clinical journey
- Enrollment of patients at UCLA
- Cell processing at Lonza

Statement of Benefit to California:

Clinical studies at UCLA Mattel Children's Hospital will further provide that medical and scientific community premier access to a cutting-edge gene therapy trial. There is a possibility that the patient(s) recruited is from California, but that is to be determined.

Source URL: https://www.cirm.ca.gov/our-progress/awards/phase-i-clinical-trial-lentiviral-gene-therapy-targeting-tcirg1-gene-infantile