

**Ex Vivo Transduction of the Human Artemis (DCLRE1C) cDNA by Lentiviral Vector AProArt into CD34+ Hematopoietic Cells for Artemis (ART)-Deficient Severe Combined Immunodeficiency (SCID)**

**Grant Award Details**

Ex Vivo Transduction of the Human Artemis (DCLRE1C) cDNA by Lentiviral Vector AProArt into CD34+ Hematopoietic Cells for Artemis (ART)-Deficient Severe Combined Immunodeficiency (SCID)

**Grant Type:** Late Stage Preclinical Projects  
**Grant Number:** CLIN1-08363  
**Project Objective:** Complete pre-clinical studies and obtain IND.

**Investigator:**

<b>Name:</b>	Jennifer Puck
<b>Institution:</b>	University of California, San Francisco
<b>Type:</b>	PI

**Disease Focus:** Blood Disorders, Immune Disease, Severe Combined Immunodeficiency, Artemis deficient (ART-SCID)  
**Human Stem Cell Use:** Adult Stem Cell  
**Award Value:** \$4,268,865  
**Status:** Closed

**Progress Reports**

**Reporting Period:** Operational Milestone #1  
[View Report](#)

**Reporting Period:** Final Operational Milestone #3  
[View Report](#)

**Grant Application Details**

**Application Title:** Ex Vivo Transduction of the Human Artemis (DCLRE1C) cDNA by Lentiviral Vector AProArt into CD34+ Hematopoietic Cells for Artemis (ART)-Deficient Severe Combined Immunodeficiency (SCID)

**Public Abstract:** **Therapeutic Candidate or Device**

Blood-forming stem cells harboring a SCID gene defect, modified to become normal by addition of a correct copy of the Artemis/DCLRE1C DNA repair gene.

**Indication**

Treatment of severe combined immunodeficiency due to defects in the Artemis/DCLRE1C gene.

**Therapeutic Mechanism**

Severe combined immunodeficiency (SCID) is characterized by absence of T and B cell immunity. Infants with SCID die of infections unless rescued by functioning hematopoietic stem cells (HSC) that grow into T and B cells. Artemis-deficient SCID patients would be best treated by correcting their own HSC so as to avoid toxicity from radiation or chemotherapy generally needed for transplants from donors other than matched siblings. Optimal treatment corrects the patient's own HSC with a lentivirus.

**Unmet Medical Need**

Current standard bone marrow transplants for SCID require chemotherapy that is harmful to Artemis SCID patients and can lead to graft rejection and graft vs host disease. Patients are tolerant to their own blood-forming stem cells, so correcting and returning them will lead to a lasting cure.

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**Major Proposed Activities**

- Manufacture sufficient pre-clinical vector for toxicity and efficacy studies and clinical grade vector for clinical trial
- Complete non-clinical toxicity studies and demonstrate ability to manufacture transduced human cells at clinical scale
- Complete non-clinical efficacy studies

**Statement of Benefit to California:** CA infants with SCID are now detected at birth by newborn screening, providing an opportunity to give immune system restoring treatment prior to onset of infections so that the infants are cured and grow up healthy. But optimal treatment for SCID caused by Artemis gene defects is elusive due to their innate sensitivity to chemotherapy used during transplants. This project will permit their safe, effective treatment. It will and make CA a world leader in gene therapy.

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**Source URL:** <https://www.cirm.ca.gov/our-progress/awards/ex-vivo-transduction-human-artemis-dclre1c-cdna-lentiviral-vector-aproart-cd34>