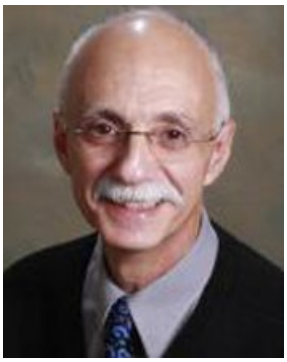
CIRM Funded Clinical Trials

Gene Transfer for Artemis-Deficient Severe Combined Immunodeficiency Using a Lentiviral Vector to Transduce Autologous CD34 Hematopoietic Stem Cells

Disease Area:	Severe Combined Immunodeficiency, Artemis deficient (ART-SCID)
Investigator:	Morton Cowan
Institution:	University of California, San Francisco
CIRM Grant:	CLIN2-10830
Award Value:	$12,000,000
Trial Sponsor:	University of California, San Francisco
Trial Stage:	Phase 1
Trial Status:	Recruiting
Targeted Enrollment:	25
ClinicalTrials.gov ID:	NCT03538899



Morton Cowan

Details:
UC San Francisco researchers aim to repair the damaged immune system of children born with severe combined immunodeficiency (SCID), a genetic blood disorder in which even a mild infection can be fatal. This trial will focus on SCID patients who have mutations in a gene called Artemis, the most difficult form of SCID to treat when using a standard bone marrow transplant from a healthy donor. The team will genetically modify the patient's own blood stem cells with a functional copy of Artemis, with the goal of creating a new blood system and restoring the health of the immune system.

Design:
Open label, single arm study.

Goal:
Safety and efficacy. Multilineage engraftment persistence and B cell reconstitution.

Source URL: https://www.cirm.ca.gov/clinical-trial/gene-transfer-artemis-deficient-severe-combined-immunodeficiency-using-lentiviral