CIRM Funded Clinical Trials

Lentiviral Gene Therapy for Infants with X-linked Severe Combined Immunodeficiency using Autologous Bone Marrow Stem Cells and Busulfan Conditioning

Disease Area:	Severe Combined Immunodeficiency, X-linked (X-SCID)
Investigator:	Stephen Gottschalk
Institution:	St. Jude Children's Research Hospital
CIRM Grant:	CLIN2-09504
Award Value:	$11,924,780
Trial Sponsor:	St. Jude Children's Research Hospital
Trial Stage:	Phase 1/2
Trial Status:	Recruiting
Targeted Enrollment:	28
ClinicalTrials.gov ID:	NCT01512888

Stephen Gottschalk

Details:
St. Jude Children's Research Hospital is teaming up with UC San Francisco to repair the damaged immune system of children born with SCID. They will genetically modify the patient's own blood stem cells, with the goal of creating a new blood system and restoring the health of the immune system.

Design:
Open label, single arm study.

Goal:
Primary: Safety and feasibility. Secondary: Efficacy; gene marking; immune reconstitution

News about this clinical trial:
Could gene therapy, with help from California's stem cell agency, treat 'bubble boy disease'?
Gene Therapy Offers Hope for Newborns with Severe Immune Disorder
Mustang Bio picks up St. Jude's 'bubble boy' gene therapy

Contact Trial Sponsor

Source URL: https://www.cirm.ca.gov/clinical-trial/lentiviral-gene-therapy-infants-x-linked-severe-combined-immunodeficiency-using