Efficacy and safety of cryopreserved autologous CD34+ HSC transduced with EFS lentiviral vector encoding for human ADA gene in ADA-SCID subjects

**Disease Area:**
Severe Combined Immunodeficiency, Adenosine deaminase-deficient (ADA-SCID)

**Investigator:** Donald Kohn

**Institution:** University of California, Los Angeles

**CIRM Grant:** CLIN2-09339-A (Closed)

**Award Value:** $10,156,925

**Trial Sponsor:** Orchard Therapeutics plc

**Trial Stage:** Phase 2

**Trial Status:** Active, not recruiting

**Targeted Enrollment:** 10

**ClinicalTrials.gov ID:** NCT02999984

**Details:**
In ADA-SCID, allogeneic hematopoietic (blood) stem cell transplants from non-matched sibling donors are a high risk procedure. Additionally, the efficacy of chronic enzyme replacement therapy is uncertain in the long-term. A team at UCLA is using a patient’s own blood stem cells to try and rebuild the damaged immune systems of patients with ADA-SCID. They will use what’s called a lentiviral vector to deliver genetic material into the blood stem cells, correcting the genetic flaw that causes SCID. It’s hoped this will create a new blood system and a healthy immune system. Preliminary data indicates that OTL-101 - a stem cell gene therapy developed by UCLA and Orchard Therapeutics Limited - may significantly improve outcomes compared to available therapies.

**Design:**
Comparability of cryopreserved product versus fresh product.

**Goal:**

**Updates:**
Breakthrough Therapy Designation. Rare Pediatric Designation. Early evidence of safety and clinical efficacy in all treated patients.

**News about this clinical trial:**
Pioneering stem cell gene therapy cures infants with bubble baby disease
Study analyzes safety and effectiveness of stem cell gene therapy for bubble baby disease
Orchard Therapeutics announces that OTL-101 has received a Rare Paediatric Disease Designation