

Gene Therapy for Adenosine Deaminase Severe Combined Immune Deficiency Using Peripheral Blood and EFS ADA Vector

NCT05432310

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	University of California, Los Angeles
Enrollment	20 participants

Key Eligibility Criteria

Inclusion (14)

- All subjects must fulfill the following criteria to be included in the study:
- Provision of written informed consent prior to any study related procedures. In this study consent must be provided by the parents/legal guardians and, where applicable according to local laws, a signed assent from the child,
- Subjects e30 days of age,
- With a diagnosis of ADA-SCID based on:
- Evidence of ADA deficiency, defined as:
... and 9 more (see full listing online)

Exclusion (40)

- Subjects will not be eligible for the study if any of the following criteria is fulfilled:
- Ineligible for autologous HSCT as per clinical site criteria
- Other conditions which in the opinion of the Principal Investigator and/or Co Investigators, contraindicate the mobilization of peripheral blood or the leukapheresis process, the administration of busulfan and the infusion of transduced cells, or which indicate an inability of the subject or subject's parent/legal guardian to comply with the protocol
- Hematologic abnormality, defined as:
- Anemia (Hb <8.0 g/dl).
... and 35 more (see full listing online)

Locations (1 total)

University of California, Los Angeles (UCLA), Los Angeles, California, United States