

# Lentiviral Gene Transfer for Treatment of Children Older Than Two Years of Age With X-Linked Severe Combined Immunodeficiency (XSCID)

NCT01306019

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<b>Status</b>	RECRUITING
<b>Phase</b>	Phase 1, Phase 2
<b>Sponsor</b>	National Institute of Allergy and Infectious Diseases (NIAID)
<b>Enrollment</b>	40 participants

## Key Eligibility Criteria

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### Inclusion (46)

- A proven mutation in the common gamma chain gene as defined by direct sequencing of patient DNA
  - No available HLA matched sibling donor as determined before enrollment. (HLA typing will be performed prior to enrollment)
  - Must be between 2 and 50 years of age and weigh greater than or equal to 10 kg
  - If previously transplanted, must be greater than or equal to 18 months post HSCT
  - Expected survival of at least 120 days.
- ... and 41 more (see full listing online)

### Exclusion (4)

- Any current or pre-existing hematologic malignancy
- Documented HIV-1 infection
- Documented active Hepatitis B infection
- Childhood malignancy (occurring before 18 years of age) in the patient or a first degree relative, or previously diagnosed known genotype of the subject conferring a predisposition to cancer (no DNA or other testing for cancer predisposition genes will be performed as part of the screen for this protocol)

## Locations (1 total)

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National Institutes of Health Clinical Center, Bethesda, Maryland, United States