

Gene Therapy for ADA-SCID Using an Improved Lentiviral Vector (IvIv-ADA)

NCT03645460

Status	RECRUITING
Phase	Not Applicable
Sponsor	Shenzhen Geno-Immune Medical Institute
Enrollment	10 participants

Key Eligibility Criteria

Inclusion (9)

- Diagnosis of classical ADA-SCID based on:
- A proven defective adenosine deaminase (ADA) gene as defined by direct sequencing of patient DNA.
- T-cell immune deficiency defined as one or more of the following: CD3+ autologous T cells \lt 300/ul, or less than 50% of normal value for in vitro mitogen stimulation, or absent proliferation in vitro to antigens.
- With severe infections, including but not limited to: pneumonitis; protracted diarrhea requiring total parenteral nutrition; infection with herpes viruses or adenovirus or fungus; disseminated BCG infection.
- No cytogenetic abnormalities (medullary karyotype) and no detection of main rearrangements associated with acute leukemia of children.

... and 4 more (see full listing online)

Exclusion (1)

- None

Locations (2 total)

Shenzhen Geno-immune Medical Institute, Shenzhen, Guangdong, China
Guilin Hospital of Chinese Traditional and Western Medicine, Guilin, Guangxi, China