

# ISP-001: Sleeping Beauty Transposon-Engineered B Cells for MPS I

NCT05682144

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<b>Status</b>	RECRUITING
<b>Phase</b>	Phase 1
<b>Sponsor</b>	Immusoft of CA, Inc.
<b>Enrollment</b>	11 participants

## Key Eligibility Criteria

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### Inclusion (6)

- Diagnosis of Mucopolysaccharidosis type I Hurler-Scheie or Scheie syndrome.
- Age  $\geq$  10 years at time of study registration.
- Creatinine clearance, calculated or measured directly, that is  $\geq$  60ml/min/1.73m<sup>2</sup>.
- Ejection fraction  $\geq$  40% by echocardiogram.
- Must commit to traveling to the study site for the necessary follow-up evaluations.

... and 1 more (see full listing online)

### Exclusion (8)

- Known familial inherited cancer syndrome. Suspected cases will be investigated, per the physicians discretion, using relevant genetic tests to determine presence of germline mutations.
- History of B cell related cancer, EBV lymphoproliferative disease or autoimmune disorders.
- Evidence of active graft-vs-host disease.
- Underwent a previous hematopoietic stem cell transplant (HSCT).
- Requirement for systemic immune suppression.

... and 3 more (see full listing online)

## Locations (2 total)

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UCSF Benioff Children's Hospital Oakland, Oakland, California, United States

University of Minnesota, Minneapolis, Minnesota, United States