

# Phase 1 Study of GC1130A in Patients With Sanfilippo Syndrome Type A (MPS IIIA)

NCT06567769

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Status	RECRUITING
Phase	Phase 1
Sponsor	GC Biopharma Corp
Enrollment	9 participants

## Key Eligibility Criteria

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### Inclusion (2)

- Participants with documented MPS IIIA diagnosis
- Participants aged e 12 months and d 18 years

### Exclusion (5)

- Participants with significant non-MPS IIIA related central nervous system impairment
- Participants with previous complication from intraventricular drug administration
- Participants with contraindications for MRI scans and for neurosurgery
- Participants that received treatment with any investigational drug or a device intended as a treatment for MPS IIIA within 30 days or 5 half-lives prior to the study
- Participants that received a hematopoietic stem cell or bone marrow transplant or received gene therapy

## Locations (5 total)

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UCSF Benioff Children's Hospital, Oakland, California, United States  
University of Minnesota, Minneapolis, Minnesota, United States  
National Center for Child Health and Development, Setagaya City, Tokyo, Japan  
... and 2 more locations