

Mucopolysaccharidosis VII Disease Monitoring Program

NCT03604835

Status RECRUITING
Sponsor Ultragenyx Pharmaceutical Inc
Enrollment 50 participants

Key Eligibility Criteria

Inclusion (3)

- Diagnosis of MPS VII based on laboratory diagnosis, including either enzymatic or mutation analysis.
- Willing and able to provide written informed consent or, in the case of patients under the age of 18 (or below adult ages as defined by local laws and regulations) or patients >18 years of age who have cognitive deficiencies, provide written assent (if required) and written informed consent by a legally authorized representative after the nature of the DMP has been explained, and prior to any research-related procedures.
- Willing to comply with DMP visit schedule.

Exclusion (1)

- Concurrent participation in other pharmaceutical company-sponsored interventional clinical trial unless approved by Ultragenyx.

Locations (14 total)

Children's Hospital of Orange County, Orange, California, United States
Children's National Health System, Washington D.C., District of Columbia, United States
Ann & Robert H. Lurie Children's Hospital of Chicago, Chicago, Illinois, United States
... and 11 more locations