A Study to Determine the Efficacy and Safety of Tividenofusp Alfa (DNL310) vs Idursulfase in Pediatric and Young Adult Participants With Neuronopathic (nMPS II) or Non-Neuronopathic Mucopolysaccharidosis Type II (nnMPS II)

NCT05371613

Status	RECRUITING
Phase	Phase 2, Phase 3
Sponsor	Denali Therapeutics Inc.
Enrollment	63 participants

Key Eligibility Criteria

Inclusion (3)

- Participants aged e2 to \<6 years (Cohort A) or e6 to \<26 years (Cohort B)
- Confirmed diagnosis of MPS II (for Cohort A, nMPS II; for Cohort B, nnMPS II)
- Have no history of treatment with enzyme replacement therapy (ERT) OR not have received continuous ERT for 4 months prior to screening OR be on maintenance ERT and have tolerated idursulfase for a minimum of 4 months prior to screening

Exclusion (5)

- Have a documented mutation of other genes or genetic diagnosis accounting for developmental delay
- Previously received an iduronate 2-sulfatase (IDS) gene therapy or stem cell therapy
- Received any CNS-targeted MPS ERT within 6 months prior to screening
- Have a contraindication for lumbar punctures and/or magnetic resonance imaging (MRI)
- Participated in any other investigational drug study or used an investigational drug within 60 days prior to screening or intend to receive another investigational drug during the study

Locations (32 total)

UCSF Benioff Children's Hospital Oakland, Oakland, California, United States
Ann and Robert H Lurie Children's Hospital of Chicago, Chicago, Illinois, United States
Hackensack University Medical Center, Hackensack, New Jersey, United States
... and 29 more locations