

MT2021-29: Evaluation of intravenous laronidase pharmacokinetics before and after hematopoietic cell transplantation in patients with mucopolysaccharidosis type IH

Status: Recruiting

Eligibility Criteria

Age: Up to 18 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

- between 0 to 3 years of age - meet protocol specific eligibility criteria for allogeneic HCT for MPS IH - planning to receive laronidase both pre and post-transplant in an inpatient setting as part of standard-of-care treatment. Virtually all patients with MPSIH being considered for transplantation at the University of Minnesota are already receiving enzyme infusions, and it is standard practice to continue to give enzyme infusions to 8 weeks post-transplant. Therefore, participation will not modify the treatment course

Exclusion Criteria:

- patient's parent/ legal guardians are unable to provide informed consent.

Conditions & Interventions

Conditions:

Rare Diseases, Cancer

Keywords:

Hematopoietic Cell Transplantation

More Information

Description: This is a prospective, observational multicenter study to collect blood from patients with mucopolysaccharidosis type IH (MPS-IH) undergoing laronidase therapy and a stem cell transplant.

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IRB

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