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

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: Up to 18 years old
This study is NOT accepting healthy 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: In this study, the researchers are collecting blood samples to learn more about laronidase treatment in children that receive a hematopoietic cell transplantation. The laronidase dose regimens used after a hematopoietic cell transplantation may differ from those administered before. This study will establish the basis for determining if there is a need to adjust laronidase dosing regimens after receiving a hematopoietic cell transplantation.
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