

MT2023-31: A multi-center, randomized, active controlled clinical trial to evaluate the efficacy and safety of OTL-203 in subjects with mucopolysaccharidosis type I, Hurler syndrome (MPS-IH) compared to standard of care with allogeneic hematopoietic stem cell transplantation (allo-HSCT)

Status: Recruiting

Eligibility Criteria

Sex: Male or Female

Age Group: Up to 18 years old

This study is NOT accepting healthy volunteers

Inclusion Criteria:

- at least 28 days old to no more than 30 months old - confirmed laboratory diagnosis of MPS-IH - evidence of altered GAG metabolism - see link to clinicaltrials.gov for complete inclusion and exclusion criteria

Exclusion Criteria:

- previous allo-HSCT or gene therapy - diagnosis of HIV, Hepatitis B, Hepatitis C, or Mycoplasma - history of uncontrolled seizures - contraindications for MRI scans - study staff will review additional exclusion criteria

Conditions & Interventions

Interventions:

Genetic: Active Comparator: Allo-HSCT, Genetic: Experimental: OTL-203

Conditions:

Rare Diseases

Keywords:

Hurler syndrome, MPS-IH, mucopolysaccharidosis type I

More Information

Description: This research study is designed to compare a new gene therapy, known as OTL-203 (study drug), with a standard treatment called "allogeneic hematopoietic stem cell transplant" (allo-HSCT), to find out which is better for the treatment of MPS-IH.

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IRB

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