MT2021-24: A Phase I Open Label Study to Evaluate the Safety and Tolerability of ISP-001 in Adult Patients with Mucopolysaccharidosis Type I Hurler-Scheie and Scheie

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: 18 years and over
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- diagnosis of Mucopolysaccharidosis type I Hurler-Scheie or Scheie syndrome - creatinine clearance, calculated or measured directly, that is greater than 60ml/min/1.73m2 - ejection fraction at least 40% by echocardiogram - must agree to stay <45-minute drive from the study site for a minimum of 5 days after cell infusion. - must commit to traveling to the study site for the necessary follow-up evaluations.

Exclusion Criteria:

- known family inherited cancer syndrome - had a previous hematopoietic stem cell transplant (HSCT) - any medical condition likely to interfere with assessment of safety or efficacy of the study treatment (study staff will review)

Conditions & Interventions

Interventions:
Biological: Autologous Plasmablasts (B cells)
Conditions:
Rare Diseases
Keywords:
Mucopolysaccharidosis IH/S, Mucopolysaccharidosis IS

More Information

Description: The purpose of the study is to determine the safety and effectiveness of a new procedure to treat Mucopolysaccharidosis Type I Hurler-Scheie and Scheie (MPS I). This procedure involves collecting some white blood cells (termed “B cells”) and growing them outside of the body in a laboratory. While the cells are in the lab, the B cells will be changed to produce more of the IDUA that is missing. This process is called “genetic modification.” The newly modified B cells are then infused back into the participant.
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