MT2019-06: A Phase 3 Study Evaluating Gene Therapy by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with the LentiGlobin BB305 Lentiviral Vector in Subjects with Sickle Cell Disease.

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: Not specified
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- must be 2 to 50 years old - diagnosis of Sickle Cell Disease - weigh a minimum of 6 kg (13.2 pounds) - treated and followed for at least the past 24 months - experienced at least 4 protocol-defined VOEs in the past 24 months - experienced HU failure at any point in the past or must have intolerance to HU - female and male subjects of childbearing potential agree to use 1 method of highly effective contraception from starting the study to at least 6 months after drug product infusion.

Exclusion Criteria:

- if allogeneic hematopoietic stem cell transplantation (allo-HSCT) is medically appropriate and a willing, human leukocyte antigen (HLA)-matched related hematopoietic stem cell donor is available - unable to receive a transfusion - prior allogeneic transplant or gene therapy - prior or current malignancy or immunodeficiency disorder, except cured tumors such as squamous cell carcinoma of the skin - women who are pregnant or breast feeding - additional exclusion criteria (study staff will review)

Conditions & Interventions

Conditions:
Blood Disorders
Keywords:
SCD, Sickle Cell Disease

More Information

Description: The purpose of this study is to evaluate the safety and ability of a transplant with your own gene modified stem cells (autologous stem cell transplant) to treat sickle cell disease. The goal is to determine if a sufficient amount of hemoglobin that prevents red blood sickling can be produced after the gene modified stem cells are returned to your body. This study may provide information on the potential usefulness of bb1111 for treatment of sickle cell disease
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