

MT2022-41 A Phase 1/2 Study Evaluating the Safety and Efficacy of a Single Dose of Autologous CD34+ Base Edited Hematopoietic Stem Cells (BEAM-101) in Patients with Sickle Cell Disease and Severe Vaso-Occlusive Crises (BEACON Trial)

Status: Recruiting

Eligibility Criteria

Sex: Male or Female

Age Group: 18 years and over

This study is NOT accepting healthy volunteers

Inclusion Criteria:

- 18 to 35 years old - documented diagnosis of sickle cell disease with specific genotypes (study staff will review) - disease is severe

Exclusion Criteria:

- HbF levels >20%, obtained at the time of screening on or off hydroxyurea therapy - previous transplant - history of an overt stroke

Conditions & Interventions

Conditions:

Rare Diseases, Blood Disorders

Keywords:

Clinics and Surgery Center (CSC), SCD, Sickle Cell Disease

More Information

Description: BEAM-101 is an experimental new therapy being developed for treating people with SCD and vaso-occlusive crises. The goal of this study is to see if BEAM-101 is safe and effective for people in the study. The study sponsor and study doctors would also like to see if individuals who are treated with BEAM-101 require fewer blood transfusions and experience fewer vasoocclusive crises requiring hospitalization, compared to before they received BEAM-101. This study will also measure the levels of fetal hemoglobin along with measures that assess quality of life and ability to function following treatment with BEAM-101.

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IRB

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