

## 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

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**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

**Interventions:**

Biological: BEAM-101

**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.

**Study Contact:** Ashish Gupta - gupta461@umn.edu

**Principal Investigator:** Ashish Gupta

**IRB**

**Number:** STUDY00017341

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