

Hematopoietic Stem Cell BCL11A Enhancer Gene Editing for Severe β Hemoglobinopathies

NCT06647979

Status	RECRUITING
Phase	Phase 1
Sponsor	Daniel Bauer
Enrollment	10 participants

Key Eligibility Criteria

Inclusion (16)

- Diagnosis of either a) sickle cell disease with genotype HbSS, HbS/B0 thalassemia, HbSD, or HbSO, or b) transfusion-dependent β thalassemia
- Age 13-40 years.
- Clinically severe disease, defined as: For sickle cell disease, the presence of one or more of the following clinical complications: i) Minimum of two episodes of acute chest syndrome (ACS) in the 2 years before study entry. ii) History of three or more episodes of severe pain events requiring a visit to a medical facility and treatment with parenteral opioids in the 2 years before study entry. For β thalassemia patients: i) At least 100 mL/kg/year or 10 units/year of blood transfusions, on an annualized basis for the two years preceding enrollment.
- Adequate hematologic parameters including:
- White blood cell (WBC) count within the range of 2.5 - 25.0 x 10⁹ /L
- ... and 11 more (see full listing online)

Exclusion (20)

- Subjects who have concomitant condition or illness including, but not limited to:
- Uncontrolled infection, such as current febrile illness, infection requiring parenteral antibiotics, or systemic fungal infection.
- Active malignancy.
- Active complication of underlying hemoglobinopathy that would place the patient at unacceptable risk for participation, in the judgment of the Investigators.
- Major surgery in the past 30 days.
- ... and 15 more (see full listing online)

Locations (1 total)

Boston Children's Hospital, Boston, Massachusetts, United States

<https://clinicaltrials.gov/study/NCT06647979>

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