Evaluation of Efficacy and Safety of a Single Dose of CTX001 in Participants With Transfusion-Dependent ²Thalassemia and Severe Sickle Cell Disease

NCT05477563

Status	RECRUITING
Phase	Phase 3
Sponsor	Vertex Pharmaceuticals Incorporated
Enrollment	26 participants

Key Eligibility Criteria

Inclusion (10)

- Participants with TDT and SCD:
- Eligible for autologous stem cell transplant as per investigator's judgment.
- Participants with TDT:
- Diagnosis of TDT as defined by:
- Documented homozygous ²thalassemia or compound heterozygous ²thalassemia including ²thalassemia/hemoglobin E (HbE). Participants can be enrolled based on historical data, but a confirmation of the genotype using the study central laboratory will be required before busulfan conditioning

... and 5 more (see full listing online)

Exclusion (9)

- Participants with TDT and SCD:
- A willing and healthy 10/10 human leukocyte antigen (HLA)-matched related donor is available per investigator's judgement
- Prior hematopoietic stem cell transplant (HSCT)
- Clinically significant and active bacterial, viral, fungal, or parasitic infection as determined by the investigator
- Participants with TDT:

... and 4 more (see full listing online)

Locations (6 total)

New York Presbyterian Hospital - Morgan Stanley Children's Hospital, New York, New York, United States
Levine Children's Hospital - Hematology, Charlotte, North Carolina, United States
TriStar Medical Group Children's Specialists - Pediatric Oncology, Nashville, Tennessee, United States
... and 3 more locations