Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of Pociredir

Status: RECRUITING

Eligibility Criteria

Age: 18 years to 65 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Key

Inclusion Criteria:

* Participant is 18 to 65 years of age, inclusive at the time informed consent is obtained. * Documented SCD at the time of screening (S/S, S/β0, S/β+, and S/C only) as confirmed through review of medical records or HPLC. * Participants who meet at least one the following criteria: 1. ≥4 to 10 episodes of SCD pain crisis over 12 months, or ≥2 to 5 over 6 months prior to screening 2. ≥2 episodes of SCD pain crisis plus at least one of the following over previous 12 months: i) Acute chest syndrome (ACS) ii. Hepatic or splenic sequestration iii. Priapism 3. ≥2 of the following events over the previous 12 months:i. ACS ii. Hepatic or splenic sequestration iii. Priapism 4. Tricuspid regurgitant jet velocity (TRV) ≥ 3.0 meter/second(m/s) OR TRV ≥ 2.5 m/s + N-terminal pro b-type natriuretic peptide (NT-proBNP) plasma level ≥ 160 picogram per milliliter; OR documented ongoing pulmonary hypertension diagnosed from previous echocardiogram or right-sided heart catheterization with mean pulmonary artery pressure \> 25 millimeter of mercury; 5. SCD-related chronic kidney disease (CKD) 6. Meet medical criteria to receive (e.g., post-cerebrovascular accident) but are contraindicated for chronic transfusions (e.g., alloimmunization, transfusion reactions) * Previous experience with Hydroxyurea (HU) but have shown to be unresponsive and/or intolerant or ineligible AND * Previous experience with a stable dose of voxelotor, crizanlizumab, and/ or L-glutamine but have shown to be unresponsive and/or intolerant or ineligible * Per Investigator's recommendation, participants may continue crizanlizumab and/or L-glutamine but must be on a stable dose for at least 6 months * HbF ≤ 20% of total Hb * Total Hb ≥ 5.5 g/dL and ≤ 12 g/dl (males) or ≤ 10.6 g/dl (females) at screening. * Participant must meet both of the following laboratory values at screening: 1. Absolute neutrophil count ≥ 1.5 × 10\^9 per liter (/l) 2. Platelets ≥ 80 × 10\^9/l 3. Absolute reticulocyte count at screening ≥ 100 x 10\^9/l. Key

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Exclusion Criteria:

* Sickle cell complication requiring care from a medical provider in hospital or emergency care setting in the 14 days prior to starting study drug. * History of bone marrow transplant or human stem cell transplant or gene therapies. * • Participants with a history of severe renal disease defined as estimated glomerular filtration rate \< 30 mL/min/1.73m\^2. Participants on dialysis of any kind are excluded. * Participants receiving regularly scheduled transfusions or therapeutic phlebotomies, or any participant who has been transfused within 60 days prior to initiating study drug. * Participant with active malignancy, or history of cancer (except for squamous cell skin cancer, basal cell skin cancer, and stage 0 cervical carcinoma in situ, with no recurrence for the last 5 years), or has an immediate family member with known or suspected familial cancer syndrome. Known presence of a chromosomal abnormality or genetic mutation that may put the participant at an increased risk of myelodysplastic syndrome (MDS) or acute myeloid leukemia (AML). * Participant currently on HU, or have received HU, within 60 days prior to initiating study drug.

Conditions & Interventions

Interventions:
DRUG: Pociredir oral capsule(s)
Conditions:
Sickle Cell Disease, Sickle Cell Anemia
Keywords:
Sickle Cell Disease, Sickle Cell Anemia, Pharmacokinetics, Pharmacodynamics, Pociredir, Open label

More Information

Contact(s): Call Center - clinicaltrials@fulcrumtx.com
Principal Investigator:
Phase: PHASE1
IRB
Number:
System ID: NCT05169580

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