A Study to Determine the Efficacy and Safety of Luspatercept in Adult Participants and to Evaluate the Safety and Pharmacokinetics in and Adolescent Participants With Alpha (α)-Thalassemia

NCT05664737

Status	RECRUITING
Phase	Phase 2
Sponsor	Bristol-Myers Squibb
Enrollment	189 participants

Key Eligibility Criteria

Inclusion (8)

- Adult participante 18 years with documented diagnosis of A-Thal HbH disease with Transfusion dependence defined as:.
- TD participant: e 6 RBC units during the 24 weeks prior to randomization.
- NTD participant:\< 6 RBC units during the 24 weeks prior to randomization(transfusion due to conditions other than A-Thal will not be considered)and, RBC transfusion-free during at least 8 weeks prior to randomization(unless transfusion was required to treat an acute medical condition other than A-Thal) and, mean baseline Hb d 10 g/dL, based on a minimum of 2 measurements e 1 week apart within 4 weeks prior to randomization; hemoglobin values within 21 days post-transfusion will be excluded.
- Adult participant has Eastern Cooperative Oncology Group (ECOG) 34 score of 0 or 1.
- Adolescent participant 12 years to \< 18 years with documented diagnosis of A-Thal HbH disease with transfusion dependence defined as:.

... and 3 more (see full listing online)

Exclusion (8)

- Medical Conditions: Diagnosis of A-ThalTrait, Hb Bart hydrops, ATRx A-Thal, hemoglobin S/²thalassemia, myelodysplasia subtype anemia, or with HbE homozygous beta gene mutation. Anemia related to nutritional deficiency, anemia of chronic disease, autoimmune hemolytic anemia, or any other hemolytic anemias. Undergone episodes of hemolysis not related to A-Thal within the 8 weeks prior to randomization.
- Participant has deep vein thrombosis (DVT), stroke or other thromboembolic event(s) (except clogged indwelling catheter) requiring medical intervention d 24weeks prior to randomization.
- Participant has uncontrolled hypertension. Controlled hypertension for this protocol is considered: blood pressure value corresponding to dGrade 1 according to NCI CTCAE Version 5.0. with or without pharmacological treatment.
- Reproductive Status: Women who are pregnant, plan to get pregnant during the study, or who are breastfeeding.
- Prior/Concomitant: Undergone HSCTs or gene therapy (candidates for HSCT or gene therapy with waiting period of e 12 months are eligible).

... and 3 more (see full listing online)

Locations (36 total)

Local Institution - 0008, Halifax, Nova Scotia, Canada
Sun Yat-sen Memorial Hospital, Sun Yat-Sen University, Guangzhou, GD, China
Nanfang Hospital of Southern Medical University, Guangzhou, GD, China
... and 33 more locations