



A phase 3, double-blind, randomized, placebo-controlled, multicenter study evaluating the efficacy and safety of mitapivat in subjects with non-transfusion-dependent alpha (α)- or beta (β)-thalassemia

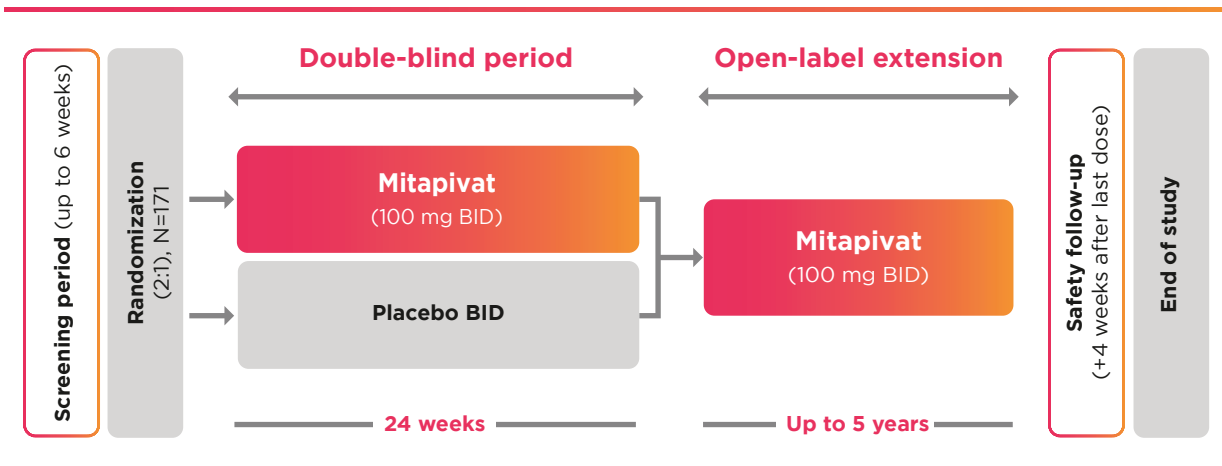
Primary endpoint

Hemoglobin (Hb) response, defined as a ≥ 1.0 g/dL increase in average Hb concentration from Week 12 through Week 24 compared with baseline

Key secondary endpoints

- Change from baseline in average Functional Assessment of Chronic Illness Therapy-Fatigue (FACIT-Fatigue) subscale score from Week 12 through Week 24
- Change from baseline in average Hb concentration from Week 12 through Week 24

Trial design



BID = twice daily

Key inclusion criteria

- ≥ 18 years of age at the time of providing informed consent
- Diagnosis of β -thalassemia with or without α -globin gene mutations, HbE/ β -thalassemia, or α -thalassemia/HbH disease
- Hb concentration ≤ 10.0 g/dL
- Non-transfusion-dependent, defined as ≤ 5 red blood cell (RBC) units during the 24-week period before randomization, and no RBC transfusions ≤ 8 weeks before providing informed consent or during the screening period

Key exclusion criteria

- Pregnant or breastfeeding
- Documented history of homozygous or heterozygous HbS or HbC
- Certain prior or current therapies
- Significant medical condition that confers an unacceptable risk to participating in the study and/or could confound the interpretation of the study data in the opinion of the investigator

For full inclusion and exclusion criteria, as well as study locations, search [ClinicalTrials.gov](https://clinicaltrials.gov) for NCT04770753.

Mitapivat is a pyruvate kinase activator approved by the US FDA for the treatment of hemolytic anemia in adults with pyruvate kinase (PK) deficiency. The safety and efficacy of mitapivat in thalassemia are under investigation and have not been established. There is no guarantee that mitapivat will receive health authority approvals or become commercially available in any country for the uses under investigation.