

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

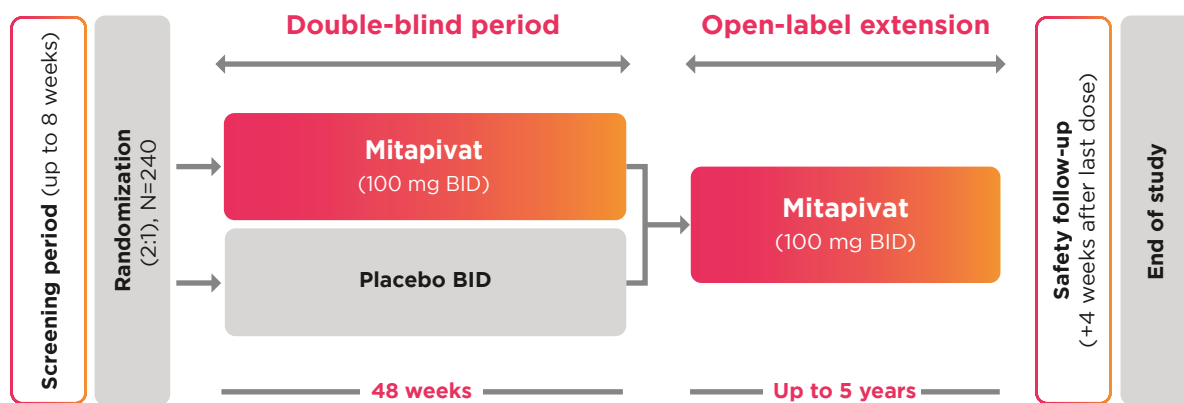
Primary endpoint

Transfusion reduction response, defined as a $\geq 50\%$ reduction in transfused red blood cell (RBC) units with a reduction of ≥ 2 units of transfused RBCs in any consecutive 12-week period through Week 48 compared with baseline

Key secondary endpoints

- $\geq 33\%$ reduction in transfused RBC units from Week 13 through Week 48 compared with baseline
- $\geq 50\%$ reduction in transfused RBC units in any consecutive 24-week period through Week 48 compared with baseline
- $\geq 50\%$ reduction in transfused RBC units from Week 13 through Week 48 compared with baseline

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
- Transfusion-dependent, defined as 6 to 20 RBC units transfused and a ≤ 6 -week transfusion-free period during the 24-week period before randomization

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 NCT04770779.

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.