THE ENERGIZE CLINICAL TRIALS

ENERGIZE-T

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

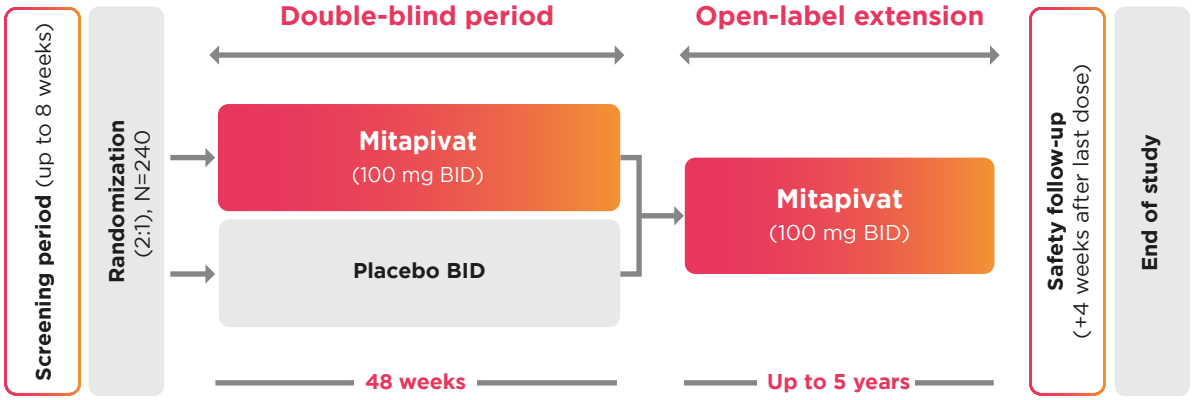
Primary endpoint

Transfusion reduction response, defined as a ≥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

- ≥33% reduction in transfused RBC units from Week 13 through Week 48 compared with baseline
- ≥50% reduction in transfused RBC units in any consecutive 24-week period through Week 48 compared with baseline
- ≥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 for NCT04770779.
Mitapivat is a pyruvate kinase (PK) activator approved by the FDA for the treatment of hemolytic anemia in adults with PK deficiency. Mitapivat is not authorized for use by the EMA or any health authority outside the United States. The safety and efficacy of mitapivat in other indications 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.

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