THE ENERGIZE CLINICAL TRIALS

ENERGIZE

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

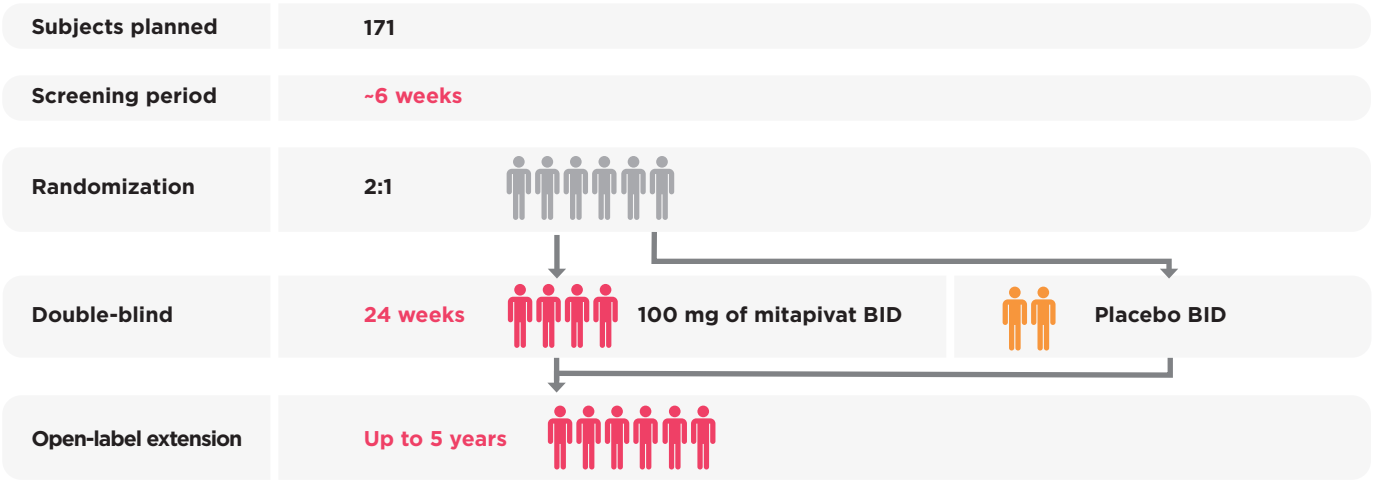
Primary endpoint

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



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 for NCT04770753

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

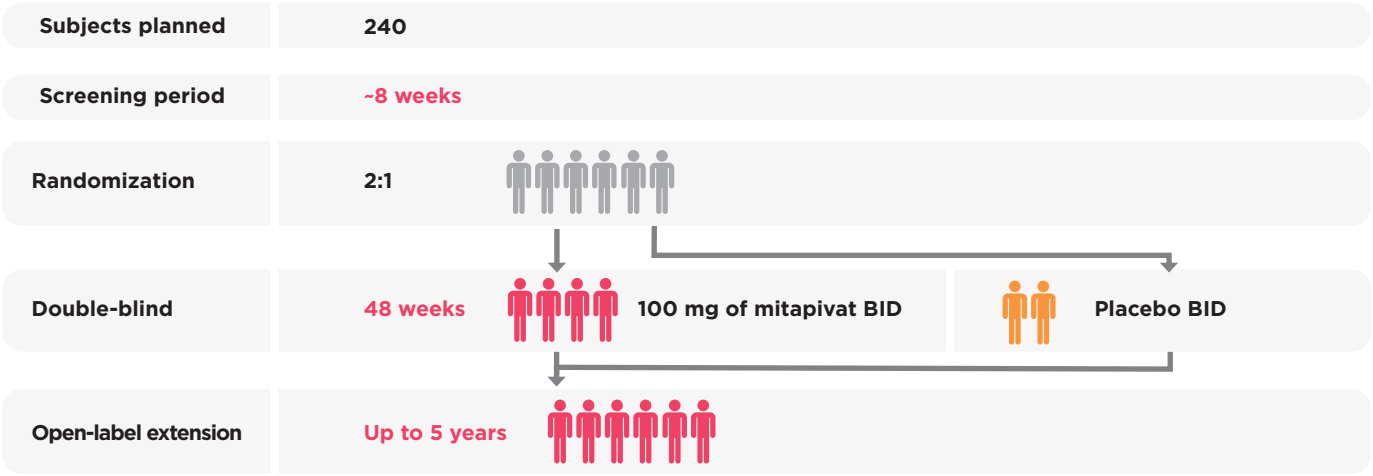
Primary endpoint

Transfusion reduction response, defined as a ≥50% reduction in transfused 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



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

US_ENG_AGIOS_AG348-C-017/018_HCP_Flashcard_v1_20210519_Protocols_27_Jan_2021

agios