Mitapivat Pediatric Clinical Trial Program

ACTIVATE-Kids™

A Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy and Safety of Mitapivat in Pediatric Subjects with Pyruvate Kinase Deficiency Who Are Not Regularly Transfused, Followed by a 5-Year Open-Label Extension Period

Key Eligibility Criteria

- Aged 1 to <18 years with central laboratory confirmation of pyruvate kinase (PK) deficiency (presence of ≥2 mutant alleles in the *PKLR* gene, of which ≥1 is a missense mutation)
- Baseline Hb: ≤10 g/dL for patients 12 to <18 years; ≤9 g/dL for patients 1 to <12 years
- ≤5 RBC transfusions in the 52 weeks prior to informed consent and no transfusions ≤12 weeks prior to first dose

Primary Endpoint

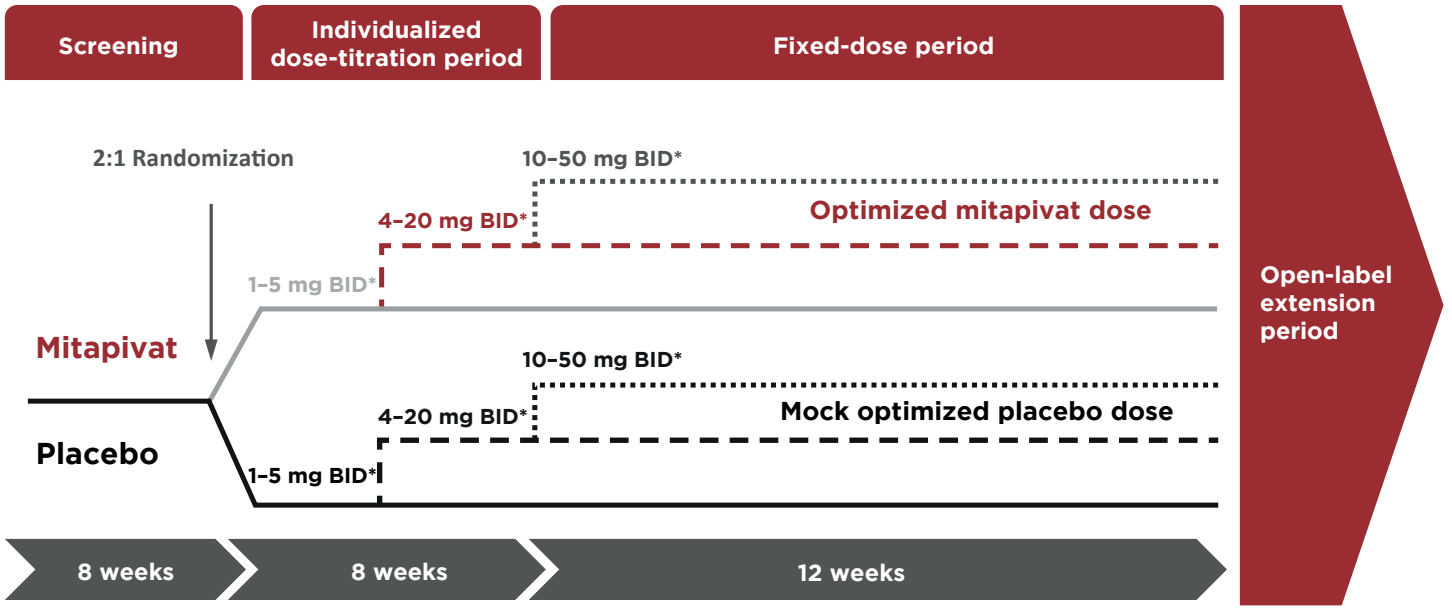
To determine effect of mitapivat on:

- Hb response, defined as a ≥1.5 g/dL increase in Hb concentration from baseline that is sustained at ≥2 scheduled assessments at Weeks 12, 16, and 20

Secondary Endpoints

To assess the effect of mitapivat on:

- Hb concentration
- Safety
- Markers of hemolysis and erythropoiesis
- Iron metabolism and overload
- Health-related quality of life
- Pharmacokinetics



*Dose of mitapivat or matched placebo based on patient's age and weight.
BID = twice daily.

Mitapivat is a 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.

For additional details about Agios study AG-348 (ACTIVATE-Kids), including the study design, study sites, or other information, please visit ClinicalTrials.gov (Identifier: NCT05175105) or contact Agios Medical Affairs at: ✉: **medinfo@agios.com;** ☎: **(+1) 833-228-8474**

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