Mitapivat Pediatric Clinical Trial Program

ACTIVATE-KidsT™

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 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)
- 6 to 26 transfusion episodes in the 52-week period before providing informed consent
- Have complete records of transfusion history for the 52 weeks before informed consent

Primary Endpoint

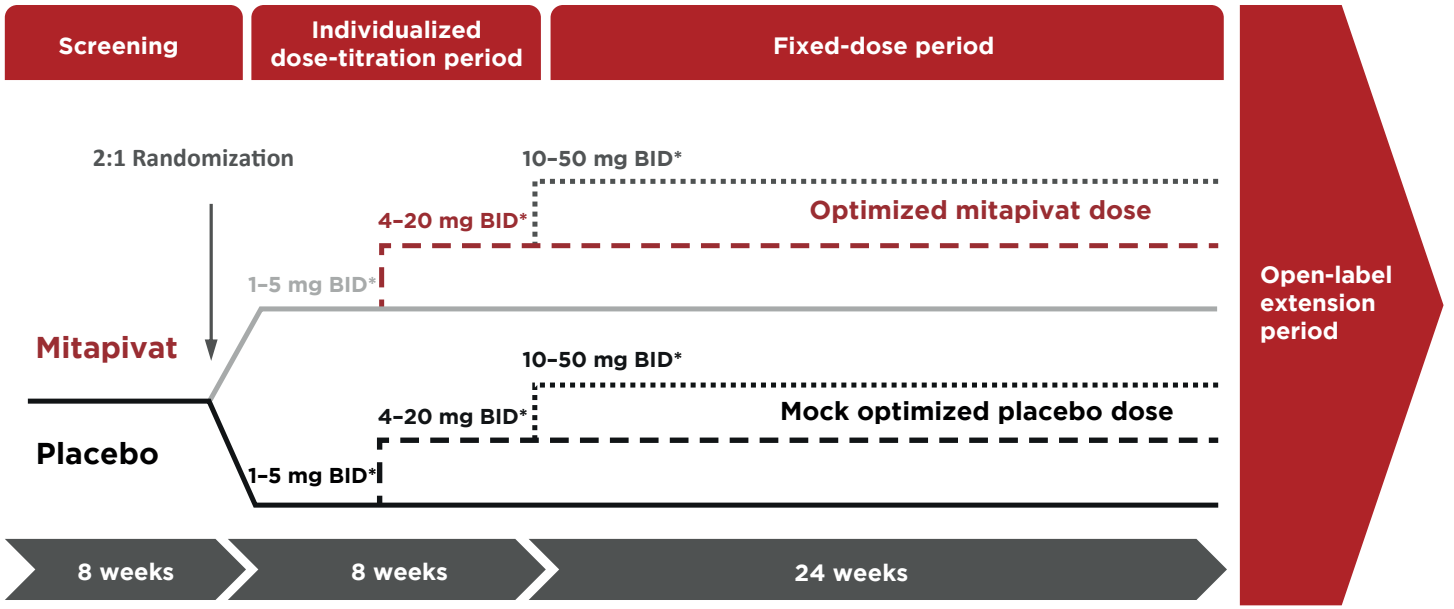
To determine effect of mitapivat on:

- Transfusion reduction response, defined as a ≥33% reduction in the total RBC transfusion volume from Week 9 through Week 32 normalized by weight and actual study drug duration compared with the historical transfusion volume standardized by weight and to 24 weeks

Secondary Endpoints

To assess the effect of mitapivat on:

- Hb concentration
- Safety
- 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.

The safety and efficacy of mitapivat in pediatrics 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 AG348-C-022 (ACTIVATE-KidsT) including the study design, study sites, or other information, please visit ClinicalTrials.gov (Identifier: NCT05144256) or contact Agios Medical Affairs at: ✉: **medinfo@agios.com;** ☎: **(+1) 833-228-8474**

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MIT-ALL-0076/2023