



**A phase 2/3, double-blind, randomized, placebo-controlled, multicenter study to evaluate the efficacy and safety of mitapivat in patients with sickle cell disease<sup>1</sup>**



## **KEY INCLUSION CRITERIA**

- Age 16 years or older, with a documented diagnosis of sickle cell disease (HbSS, HbSC, HbS/β0 thalassemia, HbS/β+ thalassemia, or other sickle cell syndrome variants)
- At least 2 and no more than 10 sickle cell pain crises in the 12 months prior to informed consent
  - Defined as acute episodes of pain, acute chest syndrome, priapism, hepatic or splenic sequestration
- If taking hydroxyurea, the hydroxyurea dose must be stable for at least 90 days before randomization

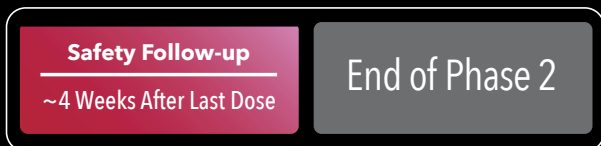
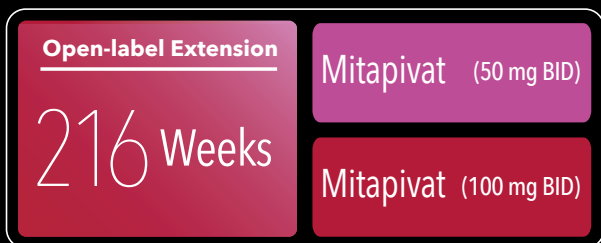
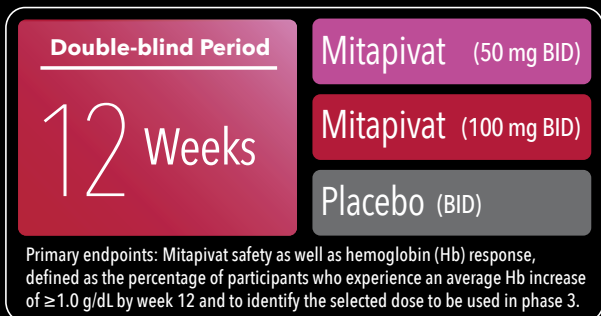
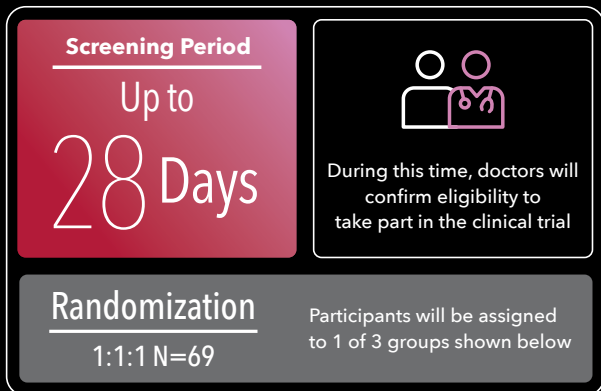


## **KEY EXCLUSION CRITERIA**

- Pregnant or breastfeeding
- Receiving regularly scheduled transfusions
- Hepatobiliary disorders, significant liver disease, gallbladder disease, or severe kidney disease
- Prior exposure to gene therapy or prior bone marrow or stem cell transplantation
- Currently receiving voxelotor, crizanlizumab, or L-glutamine
- Currently receiving treatment with hematopoietic-stimulating agents
- Taking strong CYP3A4/5 inhibitors or strong inducers or CYP3A4

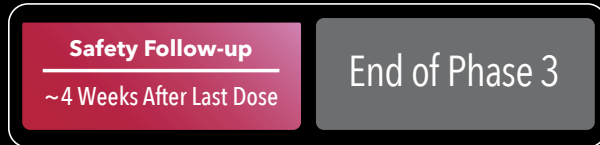
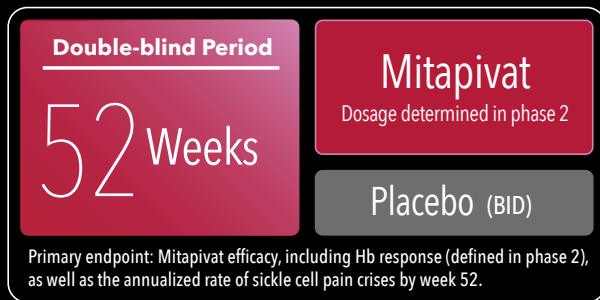
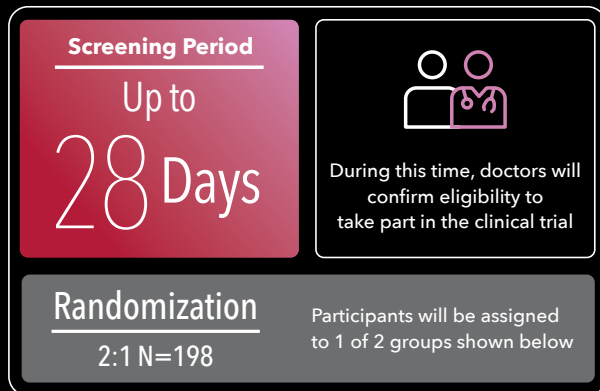
## PHASE 2

- A 3-month, placebo-controlled, dose-finding study<sup>1</sup>
- Evaluate the safety and efficacy of 2 dose levels of mitapivat in the improvement of anemia in sickle cell disease and determine the recommended phase 3 dosage<sup>1</sup>



## PHASE 3

- A 52-week, placebo-controlled, efficacy and safety study<sup>1</sup>
- Determine the effect of mitapivat on anemia and pain crises in sickle cell disease<sup>1</sup>



For additional details about Agios study AG-348-C-020 (RISE UP), including the study design, study sites, or other information, please visit [ClinicalTrials.gov](https://ClinicalTrials.gov) (Identifier: NCT05031780) or contact Agios Medical Affairs at:

✉ [medinfo@agios.com](mailto:medinfo@agios.com) ☎ 1-833-228-8474

BID=twice daily.

Reference: 1. Data on file. Agios Pharmaceuticals, Inc.

Mitapivat is a pyruvate kinase activator approved by the FDA for the treatment of hemolytic anemia in adults with pyruvate kinase (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.

