RISE UP

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[1]

KEY INCLUSION CRITERIA

- Age 16 years or older (18 years or older [France and Germany]), 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 of CYP3A4

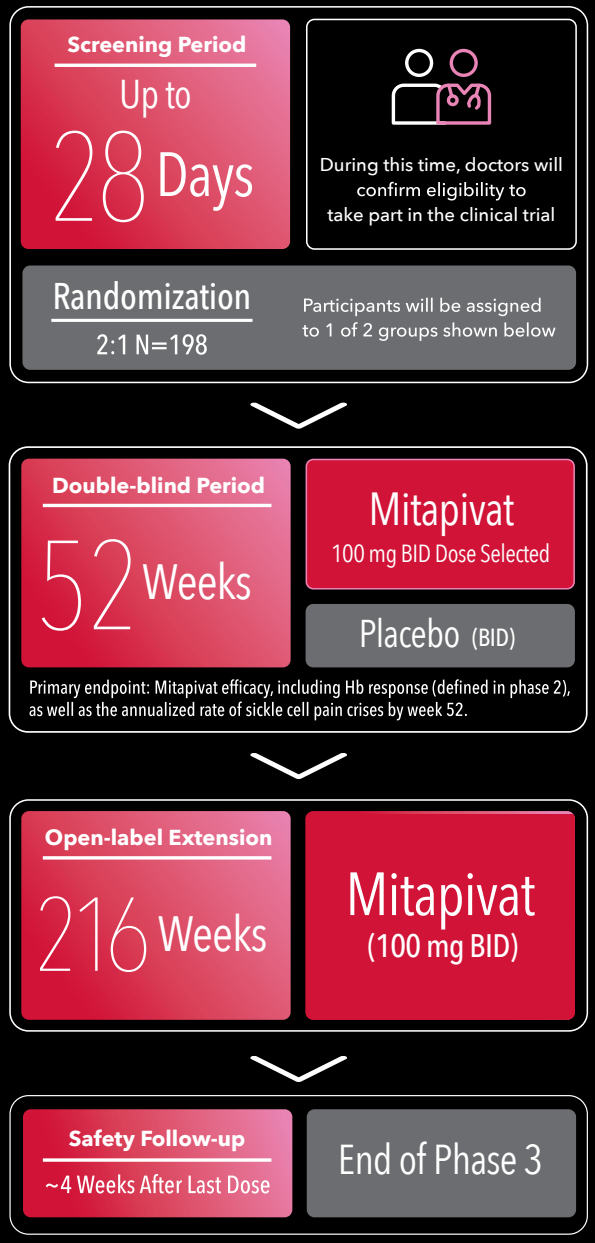
Participants will be enrolled in either phase 2 or phase 3 of RISE UP. Once enrolled, participants cannot reenroll in a different phase.[1]

PHASE 2

- A 3-month, placebo-controlled, dose-finding study[1]
- 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[1]
- **PHASE 2 ACTIVE, NOT RECRUITING**

PHASE 3

- A 52-week, placebo-controlled, efficacy and safety study[1]
- Determine the effect of mitapivat on anemia and pain crises in sickle cell disease[1]
- **PHASE 3 RECRUITING**



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** (Identifier: NCT05031780) or contact Agios Medical Affairs at:

medinfo@agios.com **1-833-228-8474**

BID=twice daily.

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

The safety and efficacy of mitapivat in sickle cell disease is under investigation and has 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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