A Phase 2, Open-label, Multicenter Study of Mitapivat in Patients With Sickle Cell Disease (SCD) and Nephropathy

Key Eligibility Criteria

- Aged ≥16 years (except in France where patients must be ≥18 years when providing informed consent)
- Hemoglobin (Hb) level 5.5–10.5 g/dL
- Confirmed SCD diagnosis (HbSS or HbS/β0-thalassemia)
- Two urine albumin creatinine ratio (ACR) results ≥100 and <2000 mg/g collected during the screening period
- Not currently receiving disease-modifying treatment for SCD other than hydroxyurea or hematopoietic stimulating agents
- If receiving hydroxyurea, the dose must be stable for ≥90 days before starting mitapivat

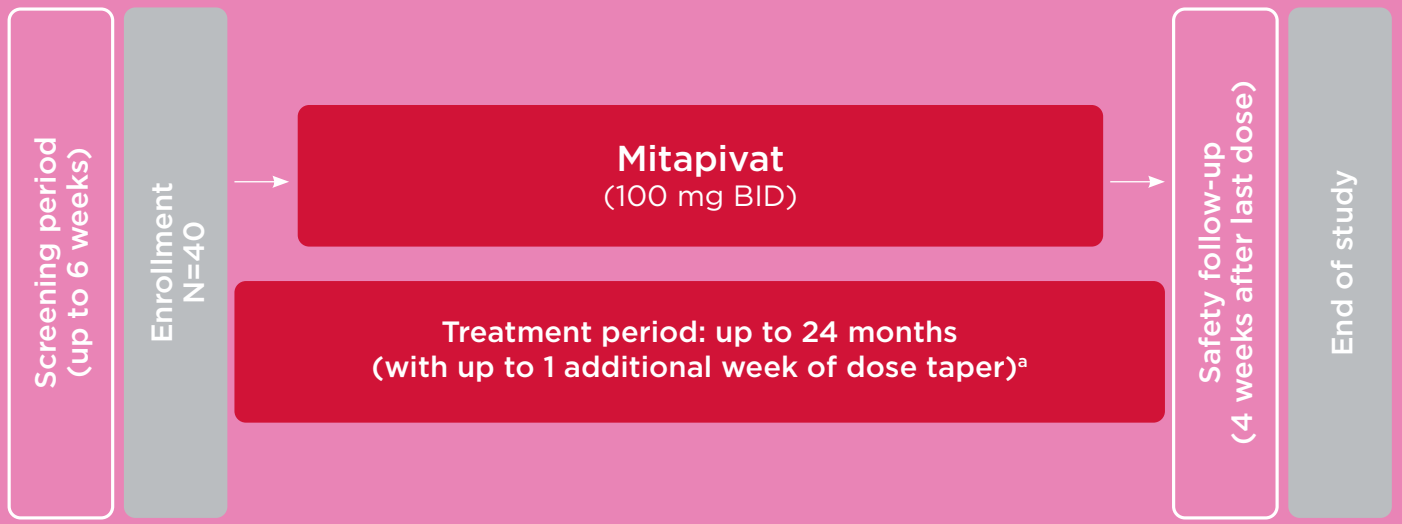
Global recruitment planned for Q3 2024

Primary Objective

- To evaluate the effect of mitapivat on ACR response, defined as ≥30% decrease in ACR from baseline to Month 6

Secondary Objectives

- To evaluate the effect of mitapivat on:
  - Cystatin C and creatinine-based eGFR (eGFRcr-cys)
  - ACR
  - Efficacy measures related to nephropathy
- To evaluate the safety of mitapivat



[a]Primary endpoint is assessed at 6 months; it is at the discretion of the Investigator and the patient whether to continue mitapivat treatment for up to 24 months

BID, twice daily

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.

For additional details about Agios study AG348-C-026 in SCD, including the study design, study sites, or other information, please visit ClinicalTrials.gov (Identifier: NCT06286046) or contact Agios Medical Affairs at: ✉: **medinfo@agios.com;** ☎: **(+1) 833-228-8474**

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