Reference number(s)
5233-A

SPECIALTY GUIDELINE MANAGEMENT

PYRUKYND (mitapivat)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Pyrukynd is indicated for the treatment of hemolytic anemia in adults with pyruvate kinase (PK) deficiency.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Initial requests:
   1. Chart notes or medical record documentation of at least one of the following:
      i. Enzyme assay demonstrating deficiency of pyruvate kinase (PK) enzyme activity.
      ii. Genetic testing demonstrating presence of at least 2 mutant alleles in the PKLR gene, of which at least 1 is a missense mutation.
   2. Chart notes or medical record documentation of blood transfusion history or hemoglobin (Hgb) levels.
B. Continuation requests: Documentation (e.g., chart notes) that the member has experienced a positive clinical response to therapy (e.g., improvement in Hgb levels, reduction in blood transfusions).

III. CRITERIA FOR INITIAL APPROVAL

Hemolytic anemia with pyruvate kinase deficiency
Authorization of 7 months may be granted for treatment of hemolytic anemia with pyruvate kinase (PK) deficiency in members 18 years of age or older when both of the following criteria are met:
A. Member meets at least one of the following:
   1. Member has a deficiency of PK enzyme activity OR
   2. Member has presence of at least 2 mutant alleles in the PKLR gene, of which at least 1 is a missense mutation.
B. Member meets at least one of the following:
   1. History of a minimum of 6 blood transfusion episodes in the past 52 weeks OR
   2. Hgb concentration less than or equal to 10.0 g/dL.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members who have hemolytic anemia with pyruvate kinase (PK) deficiency and who achieve or maintain a positive clinical response to therapy (e.g., improvement in hemoglobin levels, reduction in blood transfusions).

V. REFERENCES

1. Pyrukynd [package insert]. Cambridge, MA: Agios Pharmaceuticals, Inc.; February 2022.
2. Al-Samkari H, Galacteros F, Glenthoj A, et al. Mitapivat versus placebo for pyruvate kinase deficiency. *N Engl J Med*. 2022 Apr 14;386(15):1432-1442.
3. A Study Evaluating the Efficacy and Safety of AG-348 in Regularly Transfused Adult Participants with Pyruvate Kinase Deficiency (PKD). ClinicalTrials.gov. https://clinicaltrials.gov/ct2/show/study/NCT03559699. Published January 4, 2022. Accessed August 15, 2023.