

Specialty Guideline Management

Pyrukynd

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Pyrukynd	mitapivat

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 Indication¹

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.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Initial requests:

- Chart notes or medical record documentation of at least one of the following:
 - Enzyme assay demonstrating deficiency of pyruvate kinase (PK) enzyme activity.
 - Genetic testing demonstrating presence of at least 2 variant alleles in the pyruvate kinase liver and red blood cell (PKLR) gene, of which at least 1 is a missense variant.
- Chart notes or medical record documentation of blood transfusion history or hemoglobin (Hgb) levels.

Continuation requests:

Chart notes or medical record documentation that the member has experienced a positive clinical response to therapy (e.g., improvement in hemoglobin [Hgb] levels, reduction in blood transfusions).

Prescriber Specialties

This medication must be prescribed by or in consultation with a hematologist or specialist in pyruvate kinase deficiency.

Coverage Criteria

Hemolytic anemia with pyruvate kinase deficiency¹⁻³

Authorization of 12 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:

- Member meets at least one of the following:
 - Member has a deficiency of PK enzyme activity
 - Member has presence of at least 2 variant alleles in the pyruvate kinase liver and red blood cell (PKLR) gene, of which at least 1 is a missense variant.
- Member meets at least one of the following:
 - History of a minimum of 6 blood transfusion episodes in the past 52 weeks
 - Hemoglobin (Hgb) concentration less than or equal to 10.0 grams per deciliter (g/dL).

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 [Hgb] levels, reduction in blood transfusions).

References

1. Pyrukynd [package insert]. Cambridge, MA: Agios Pharmaceuticals, Inc.; January 2025.
2. Al-Samkari H, Galacteros F, Glenthøj A, et al. Mitapivat versus placebo for pyruvate kinase deficiency. *N Engl J Med*. 2022 Apr 14;386(15):1432-1442.
3. Glenthøj A, van Beers EJ, Al-Samkari H, et al. Mitapivat in adult patients with pyruvate kinase deficiency receiving regular transfusions (ACTIVATE-T): a multicentre, open-label, single-arm, phase 3 trial. *Lancet Haematol*. 2022 Oct;9(10):e724-e732.