CVS Caremark®

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| Reference number(s) |
| 5233-A |

# 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 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.

## 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 mutant alleles in the PKLR gene, of which at least 1 is a missense mutation.
* Chart notes or medical record documentation of blood transfusion history or hemoglobin (Hgb) levels.

### 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).

## Coverage Criteria

### 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:

* Member meets at least one of the following:
  + Member has a deficiency of PK enzyme activity
  + Member has presence of at least 2 mutant alleles in the PKLR gene, of which at least 1 is a missense mutation.
* Member meets at least one of the following:
  + History of a minimum of 6 blood transfusion episodes in the past 52 weeks
  + Hgb concentration less than or equal to 10.0 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 levels, reduction in blood transfusions).

## 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. Glenthoj 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.