Pharmacy Medical Necessity Guidelines: Pyrukynd® (mitapivat)

Effective: September 1, 2023

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	RX	Department to Review	RXUM
These pharmacy medical necessity guidelines apply to the following: ☒ Tufts Health RITogether – A Rhode Island Medicaid Plan		**Fax Numbers:** RXUM: 617.673.0988	

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION (FDA)-APPROVED INDICATIONS

Pyrukynd (mitapivat) is a pyruvate kinase activator indicated for the treatment of hemolytic anemia in adults with pyruvate kinase (PK) deficiency.

COVERAGE GUIDELINES

The plan may authorize coverage of Pyrukynd (mitapivat) for Members when the following criteria are met:

Initial Therapy

1. Documented diagnosis of pyruvate kinase deficiency

AND

2. Documentation of **one (1)** of the following:
   a. The patient has a current hemoglobin level less than or equal to 10 g/dL
   b. The patient has required a minimum of six (6) red blood cell transfusions for hemolytic anemia due to pyruvate kinase deficiency within the previous 12 months

AND

3. The patient is at least 18 years of age

AND

4. Prescribed by or in consultation with a hematologist

Reauthorization Criteria

1. Documented diagnosis of pyruvate kinase deficiency

AND

2. The patient is at least 18 years of age

AND

3. Prescribed by or in consultation with a hematologist

AND

4. Documentation the patient has experienced a therapeutic response as defined by **one (1)** of the following:
   a. At least a 1.5 g/dL increase in hemoglobin from baseline
   b. A reduction in transfusion burden (e.g., at least a 33% reduction in the number of red blood cell units transfused compared to pre-treatment experience)

LIMITATIONS

1. Initial coverage of Pyrukynd (mitapivat) will be authorized for 6 months. Reauthorization of Pyrukynd (mitapivat) will be provided for 12-month intervals.
2. Members new the plan stable on Pyrukynd (mitapivat) should be reviewed against Reauthorization Criteria.

CODES

None.

REFERENCES

1. Al-Samkari H, van Beers EJ. Mitapivat, a novel pyruvate kinase activator, for the treatment of hereditary hemolytic anemias. *Ther Adv Hematol*. 2021a; 12:1-11..
2. Al-Samkari H, Galacteros F, Glenthoj A, et al. ACTIVATE: a phase 3, randomized, multicenter, double-blind, placebo-controlled study of mitapivat in adults with pyruvate kinase deficiency who are not regularly transfused (abstract). *HemaSphere*. 2021b; 5(S2): S270.
3. Glenthoj A, van Beers EJ, Al-Samkari H, et al. ACTIVATE-T: a phase 3, open-label, multicenter study of mitapivat in adults with pyruvate kinase deficiency who are regularly transfused.

Presented at: European Haematology Association Virtual Congress; June 9 – June 17, 2021; virtual meeting. Oral presentation S271.
4. Pyrukynd (mitapivat) [prescribing information]. Cambridge, MA: Agios Pharmaceuticals, Inc.; February 2022.

APPROVAL HISTORY

September 13, 2022: Reviewed by Pharmacy & Therapeutics Committee.

- June 13, 2023: Added "Documentation of one (1) of the following: a. The Patient has a current hemoglobin level less than or equal to 10 g/dL or b. The patient has required a minimum of six (6) red blood cell transfusions for hemolytic anemia to pyruvate kinase deficiency within the previous 12 months." to Initial Therapy coverage criteria (effective September 1, 2023).

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.

Provider Services