

PHARMACY POLICY STATEMENT

Marketplace

DRUG NAME	Pyrukynd (mitapivat)
BILLING CODE	Must Use Valid NDC
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

Pyrukynd is a pyruvate kinase activator indicated for treatment of adults with hemolytic anemia caused by pyruvate kinase (PK) deficiency.¹ Pyruvate kinase deficiency impacts red blood cells causing them to break down too easily. This condition is called hemolytic anemia.¹ Hemolytic anemia is characterized by red blood cells being destroyed faster than they can be created. Pyrukynd works by reducing this cellular malfunction⁴; it was initially approved by the FDA in 2022.³

Pyrukynd (mitapivat) will be considered for coverage when the following criteria are met:

Hemolytic Anemia in adults with Pyruvate Kinase (PK) deficiency

For **initial** authorization:

1. Member must be 18 years of age or older; AND
2. Medication must be prescribed by or in consultation with a geneticist or hematologist; AND
3. Member has a diagnosis of PK deficiency confirmed by genetic testing (two or more documented mutant *PKLR* alleles, at least one of which is a missense mutation⁴); AND
4. Member has a documented baseline hemoglobin of <10.0 g/dl (regardless of sex)⁴; AND
5. Member is currently taking at least 0.8 mg oral folic acid as indicated by chart notes; AND
6. Member does NOT have any of the following:
 - a) Homozygous R479H mutation or have 2 non-missense mutations, without the presence of another missense mutation, in the *PKLR* gene⁵, nor
 - b) Moderate to severe hepatic impairment³, nor
 - c) Splenectomy currently scheduled⁵, nor
 - d) Prior bone marrow or stem cell transplant⁵, nor
 - e) Currently receiving anabolic steroids, such as testosterone⁵
7. **Dosage allowed/Quantity limits:**
 - a) **Starting Dose:** 5mg twice daily for the first four (4) weeks
 - b) **Maintenance Doses:** Titrate to 20 mg twice daily, and then to the maximum recommended dose of 50 mg twice daily, with these dose increases occurring every 4 weeks, based on assessments of Hb and transfusion requirements, as directed in prescribing information.^{2,3}
 - c) **Quantity Limit:** 58 tablets/28 days.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show normalized hemoglobin or increase of at least 1.5 g/dL Hb or clinically significant decrease in frequency of transfusions or units transfused; AND
2. Chart notes must show liver function tests within normal range³.

If all the above requirements are met, the medication will be approved for an additional 6 months.

CareSource considers Pyrukynd (mitapivat) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
4/16/2022	New policy for Pyrukynd created.

References:

1. Pyruvate kinase deficiency | Genetic and Rare Diseases Information Center (GARD) – an NCATS Program (nih.gov). Accessed April 25, 2022.
2. How PYRUKYND works. <https://www.pyrukynd.com/about-pyrukynd/how-pyrukynd-works/>. Accessed April 16, 2022.
3. Pyrukynd [package insert]. Cambridge, Massachusetts: Agios Pharmaceuticals, Inc.; 2022.
4. Al-Samkari H, van Beers EJ. Mitapivat, a novel pyruvate kinase activator, for the treatment of hereditary hemolytic anemias. *Ther Adv Hematol*. 2021;12:20406207211066070. Published 2021 Dec 21. doi:10.1177/20406207211066070
5. A Study to Evaluate Efficacy and Safety of AG-348 in Not Regularly Transfused Adult Participants With Pyruvate Kinase Deficiency (PKD). <https://www.clinicaltrials.gov/ct2/show/NCT03548220>. Accessed April 28, 2022.
6. Grace RF, Mark Layton D, Barcellini W. How we manage patients with pyruvate kinase deficiency [published correction appears in Br J Haematol. 2019 May;185(4):807]. *Br J Haematol*. 2019;184(5):721-734. doi:10.1111/bjh.15758
7. Grace RF, Barcellini W. Management of pyruvate kinase deficiency in children and adults. *Blood*. 2020;136(11):1241-1249. doi:10.1182/blood.2019000945.
8. Grace RF, Rose C, Layton DM, et al. Safety and Efficacy of Mitapivat in Pyruvate Kinase Deficiency. *N Engl J Med*. 2019;381(10):933-944. doi:10.1056/NEJMoa1902678

Effective date: 10/01/2022

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