CareSource Rx Innovations

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Aqvesme (mitapivat)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Aqvesme, approved by the FDA in 2025, is a pyruvate kinase activator indicated for the treatment of anemia in adults with alpha- or beta-thalassemia. It is the first drug approved for alpha-thalassemia and the first to be approved for non-transfusion dependent beta-thalassemia (NTDT). Other therapies are available for transfusion dependent beta-thalassemia (TDT).
The thalassemias are inherited hemoglobin disorders characterized by defective synthesis of the α-globin (α-thalassemia) or β-globin (β-thalassemia) chains of adult hemoglobin A (HbA). α-thalassemia is caused by mutations in the HBA1 and HBA2 genes. β-thalassemia is caused by mutations in the HBB gene. Clinical severity depends on the extent of globin chain imbalance based on the genotype. Manifestations are the result of ineffective erythropoiesis, hemolytic anemia and dysregulated iron homeostasis.
The active ingredient, mitapivat, was originally approved as brand name Pyrukynd for anemia due to pyruvate kinase deficiency.

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

Alpha- or Beta-Thalassemia

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a hematologist; AND
3. Member has a documented diagnosis of alpha- or beta-thalassemia; AND
4. Member meets one of the following:
   a) Hemoglobin ≤10.0 g/dL
   b) Transfusion dependence (i.e., at least 6 RBC units transfused in the past 6 months with 6 weeks or less transfusion-free period in the last 6 months); AND
5. Liver function tests have been or will be measured before starting treatment; AND
6. Member does NOT have cirrhosis (Child-Pugh Class A, B, or C)
7. **Dosage allowed/Quantity limit:** 100 mg orally twice daily. QL: 56 tablets per 28 days.

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

For **reauthorization**:
1. Chart notes must document one of the following in response to treatment:
   a) At least 1.0 g/dL increase in hemoglobin from baseline
   b) Clinically significant reduction in blood transfusions

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Aqvesme (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
01/23/2026	New policy created for Aqvesme.

References:

1. Aqvesme [prescribing information]. Agios Pharmaceuticals, Inc.; 2025.
2. Kuo KHM, Layton DM, Lal A, et al. Long-term efficacy and safety of mitapivat in non-transfusion-dependent α- or β-thalassaemia: An open-label phase 2 study. *Br J Haematol*. 2025;206(6):1764-1773. doi:10.1111/bjh.20058
3. Thalassaemia International Federation (TIF). Guidelines for the Management of α-Thalassaemia (2023). https://thalassaemia.org.cy/publications/tif-publications/
4. Thalassaemia International Federation (TIF). Guidelines for the Management of Non-Transfusion-Dependent β-Thalassaemia (3rd edition – 2023). https://thalassaemia.org.cy/publications/tif-publications/
5. Thalassaemia International Federation (TIF). Guidelines for the Management of Transfusion-Dependent β-Thalassaemia (5th edition – 2025). https://thalassaemia.org.cy/publications/tif-publications/

Effective date: 07/01/2026
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