Mass General Brigham
Health Plan

Aqvesme (mitapivat)
Effective 09/01/2026

Plan	☐ MassHealth UPPL ☒ Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☒ Pharmacy Benefit ☐ Medical Benefit		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	**Medical Benefit** **Pharmacy Benefit**	Phone: 833-895-2611 Phone: 800-711-4555	Fax: 888-656-6671 Fax: 844-403-1029
Exceptions	N/A		

Overview
Aqvesme (mitapivat) is a pyruvate kinase activator indicated for the treatment of anemia in adults with alpha- or beta-thalassemia.

Coverage Guidelines
If member is new to the plan (as evidenced by coverage effective date of less than or equal to 90 days), submission of medical records documenting that the member is currently receiving treatment with the requested drug, excluding when the product is obtained as samples or via manufacturer's patient assistance programs
OR
Authorization may be granted when all of the following criteria are met:

1. Submission of medical records (e.g., chart notes) documenting all of the following:
    a. Diagnosis of anemia with alpha or beta-thalassemia
    b. Hemoglobin is less than or equal to 10g/dL
    c. Member has symptomatic anemia or is transfusion dependent
    d. Member does not have liver disease (e.g., cirrhosis - Child Pugh Class A, B, or C; Hep B virus [HBV], Hepatitis C virus [HCV], Human Immunodeficiency virus [HIV-1 or HIV -2])
2. Both of the following:
    a. Member has not had prior exposure to gene therapy for alpha or beta thalassemia (e.g., Casgevy, Zynteglo)
    b. Member has not had prior bone marrow or stem cell transplantation
3. Member is not on concurrent treatment with any of the following:
    a. Reblozyl (luspatercept-aamt)
    b. Hematopoietic stimulating agents (e.g., Procrit, Retacrit [epoetin alfa], Aranesp [darbepoetin alfa])
    c. Pyrukynd (mitapivat)
4. Requested medication is prescribed by or in consultation with one of the following:
    a. Hematologist
    b. Hepatologist

Continuation of Therapy

Requests for reauthorization will be approved when all of the following criteria are met:

1. Submission of medical records (e.g., chart notes) documenting member has had a positive clinical response to therapy (e.g., reduction in RBC transfusion burden, improvement in fatigue, increase in Hemoglobin concentration greater than or equal to 1g/dL from baseline)
2. Requested medication is prescribed by or in consultation with one of the following:
    a. Hematologist
    b. Hepatologist
3. Member is not on concurrent treatment with any of the following:
    a. Reblozyl (luspatercept-aamt)
    b. Hematopoietic stimulating agents (e.g., Procrit, Retacrit [epoetin alfa], Aranesp [darbepoetin alfa])
    c. Pyrukynd (mitapivat)

Limitations

1. Initial approvals and reauthorizations will be granted for 12 months
2. The following quantity limits apply

Drug Name and Dosage	Quantity Limit
Aqvesme tablet	2 tablets per day

References

1. Aqvemse (mitavipat) [prescribing information]. Agios Pharmaceuticals, Inc.: Cambridge, MA; December 2025.
2. Cappellini MD, Sheth S, Taher AT, et al. ENERGIZE-T: A global, phase 3, double-blind, randomized, placebo-controlled study of mitapivat in adults with transfusion-dependent alpha- or beta-thalassemia. *Blood*. 2024;144(Suppl1):409. Abstract. Accessed January 2, 2026. ENERGIZE-T: A Global, Phase 3, Double-Blind, Randomized, Placebo-Controlled Study of Mitapivat in Adults with Transfusion-Dependent Alpha- or Beta-Thalassemia | Blood | American Society of Hematology
3. Cappellini MD, Viprakasit V, Taher AT, et al. A phase 3 trial of luspatercept in patients with transfusion-dependent β-thalassemia. *N Engl J Med*. 2020;382(13):1219-1231. doi: 10.1056/NEJMoa1910182.
4. Cappellini MD, Viprakasit V, Georgiev P, et al. Long-term efficacy and safety of luspatercept for the treatment of anaemia in patients with transfusion-dependent β-thalassaemia (BELIEVE): final results from a phase 3 randomised trial. *Lancet Haematol*. 2025;12(3):e180-e189. doi: 10.1016/S2352-3026(24)00376-4.
5. Guidelines for the management of non-transfusion-dependent β-thalassaemia. 3rd Ed. Taher AT, Musallam KM, Cappellini MD (Eds). Thalassaemia International Federation, 2023.
6. Guidelines for the management of transfusion-dependent β-thalassaemia (TDT). 5th Ed. Taher AT, Farmakis D, Porter JB, Cappellini MD, Musallam KM (Eds). Thalassaemia International Federation, 2025c.
7. Taher AT, Al-Samkari H, Adyinok Y, et al. Mitapivat in adults with non-transfusion-dependent α-thalassaemia or β-thalassaemia (ENERGIZE): a phase 3, international, randomised, double-blind, placebo-controlled trial. *Lancet*. 2025a;406(10498): 33-42. doi: 10.1016/S0140-6736(25)00635-X.
8. Tantiworawita A, Kamolsripata T, Piriyakhuntorna P, et al. Survival and causes of death in patients with alpha and beta-thalassemia in Northern Thailand. *Ann Med*. 2024;56(1):2338246. doi: 10.1080/07853890.2024.2338246.

Review History

05/13/2026 – Reviewed at May P&T. Effective 09/01/2026.