Reference number(s)
7343-A

Specialty Guideline Management
Aqvesme

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
Aqvesme	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 Indications[1]

Aqvesme is indicated for treatment of anemia in adults with alpha- or beta-thalassemia.

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:

Anemia with Alpha- or Beta-Thalassemia

Initial requests:

- Pretreatment or pretransfusion hemoglobin (Hgb) level

- Either of the following:
  - Hemoglobin electrophoresis or high-performance liquid chromatography (HPLC) results OR molecular genetic testing results, or
  - Chart notes or medical record documentation stating diagnosis of thalassemia was previously confirmed by hemoglobin electrophoresis or HPLC results, OR molecular genetic testing

Prescriber Specialties

This medication must be prescribed by or in consultation with a hematologist or specialist in the treatment of alpha- or beta-thalassemia.

Exclusions[1]

Coverage will not be provided for members who meet any of the following:

- The member has a diagnosis of homozygous or heterozygous sickle hemoglobin (HbS).
- The member has a diagnosis of homozygous or heterozygous hemoglobin C (HbC).
- The member will use the requested medication concomitantly with luspatercept-aamt (Reblozyl).

Coverage Criteria

Anemia with Alpha- or Beta-Thalassemia[1,]

Authorization of 12 months may be granted for treatment of anemia with alpha- or beta-thalassemia in members 18 years of age or older when all of the following criteria are met:

- The member has symptomatic anemia evidenced by a pretreatment or pretransfusion Hgb level less than or equal to 11 grams per deciliter (g/dL).
- The member has a diagnosis of thalassemia (beta-thalassemia [β-thalassemia] with or without alpha-globin [α-thalassemia] gene mutations, hemoglobin E [HbE]/β-thalassemia, or α-thalassemia/hemoglobin H [HbH] disease) confirmed by either of the following:
  - Hemoglobin electrophoresis or high-performane liquid chromatography (HPLC)
  - Molecular genetic testing
- For members with transfusion-dependent thalassemia, the member required at least 6 red blood cell (RBC) units to be transfused in the previous 24 weeks.

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in all members (including new members) who are currently receiving the requested medication and who are experiencing benefit from therapy as evidenced by either of the following:

- The member has achieved or maintained a reduction in red blood transfusion burden.
- The member has achieved or maintained an increase in hemoglobin from baseline.

References

1. Aqvesme [package insert]. Cambridge, MA: Agios Pharmaceuticals, Inc.; December 2025.