



Recall Alert

An update on the Cyclospora outbreak available [here](#).



U.S. FOOD & DRUG
ADMINISTRATION

Search

Menu

[Home](#) / [Drugs](#) / [News & Events for Human Drugs](#) / [FDA approves first oral treatment for anemia in thalassemia, an inherited blood disorder](#)

FDA approves first oral treatment for anemia in thalassemia, an inherited blood disorder

News & Events for Human Drugs

Meetings,
Conferences, &
Workshops

Q&A with FDA Podcast

CDER
Conversations

From Our
Perspective

Spotlight on CDER
Science

Things to Know
About...

Action

The U.S. Food and Drug Administration (FDA) approved [Aqvesme \(mitapivat\) tablets](#) to treat anemia (low levels of red blood cells) in adults with alpha- or beta-thalassemia. This is the first oral treatment option for patients with beta-thalassemia and the first drug approval for patients with alpha thalassemia.

Disease or Condition

Thalassemia is an inherited blood disorder caused by mutations in the genes responsible for producing alpha and beta globins. These globins are components of hemoglobin, a protein in red blood cells that carries oxygen. The abnormal effects on globin chain production damages the red blood cells, reducing their production and increasing their destruction (hemolysis). Some patients with thalassemia may be transfusion-dependent, requiring regular blood transfusions to manage their condition, typically every two to five weeks.

Data Supporting Aqvesme

The efficacy and safety of Aqvesme were evaluated in two multinational, randomized, double-blind, placebo-controlled clinical studies.

The ENERGIZE-T study evaluated 258 adults with transfusion-dependent thalassemia, with 171 adults receiving Aqvesme and 87 receiving placebo. Efficacy was based upon transfusion reduction response, defined as greater than 50% reduction in the number of red blood cell units transfused with a reduction of at least two units in any consecutive 12-week period between the baseline visit and Week 48. A higher proportion of patients taking Aqvesme achieved a transfusion reduction response (30%) compared with the placebo group (13%).

A second trial, the ENERGIZE study, evaluated Aqvesme in 194 adults with non-transfusion-dependent thalassemia over 24 weeks, with 130 adults receiving Aqvesme daily and 64 receiving placebo. Efficacy was based upon hemoglobin response (a measure of the improvement in anemia), defined as a ≥ 1 g/dL increase from baseline in mean hemoglobin concentration at Week 24. A higher proportion of patients taking Aqvesme achieved a hemoglobin response (42%) compared with the placebo group (2%).

Another efficacy endpoint in the ENERGIZE study assessed the mean change from baseline in fatigue-related symptoms and impacts using a patient-reported outcome instrument, the Functional Assessment of Chronic Illness Therapy-Fatigue Scale (FACIT-Fatigue). The FACIT-F total score has a range of 0 to 52, with higher scores indicating less fatigue. At baseline, the mean FACIT-F

Content current as of:

01/05/2026

Regulated

Product(s)

Drugs

Feedback

Agios Pharmaceuticals, Inc.
Exhibit 2019
Apotex v. Agios
IPR2026-00351
Ex. 2019-001

baseline visit and Week 48. A higher proportion of patients taking Aqvesme achieved a transfusion reduction response (30%) compared with the placebo group (13%).

A second trial, the ENERGIZE study, evaluated Aqvesme in 194 adults with non-transfusion-dependent thalassemia over 24 weeks, with 130 adults receiving Aqvesme daily and 64 receiving placebo. Efficacy was based upon hemoglobin response (a measure of the improvement in anemia), defined as a ≥ 1 g/dL increase from baseline in mean hemoglobin concentration at Week 24. A higher proportion of patients taking Aqvesme achieved a hemoglobin response (42%) compared with the placebo group (2%).

Another efficacy endpoint in the ENERGIZE study assessed the mean change from baseline in fatigue-related symptoms and impacts using a patient-reported outcome instrument, the Functional Assessment of Chronic Illness Therapy-Fatigue Scale (FACIT-Fatigue). The FACIT-F total score has a range of 0 to 52, with higher scores indicating less fatigue. At baseline, the mean FACIT-F score was approximately 36. Patients treated with Aqvesme had a mean increase in the FACIT-F total score of 4.9 compared to a mean increase of 1.5 in patients taking placebo.

The recommended dosage for Aqvesme is 100 mg orally twice daily with or without food.

Safety Information

Aqvesme is only available through a restricted program called the Aqvesme Risk Evaluation and Mitigation Strategies (REMS) because of the risk of liver toxicity observed in the clinical trials.

The most common side effects of Aqvesme are headache and insomnia. More safety information is available in the prescribing information.

Feedback

Was this page helpful? *

☐ Yes

☐ No

Submit

 An official form of the United States government. Provided by [Touchpoints](#)

[FDA Archive](#)

[Visitor Information](#)

[FOIA](#)

[About FDA](#)

[Website Policies / Privacy](#)

[HHS.gov](#)

[Accessibility](#)

[No FEAR Act](#)

[USA.gov](#)

[Vulnerability Disclosure Policy](#)

Contact FDA



1-888-INFO-FDA (1-888-463-6332)



Document title: FDA approves first oral treatment for anemia in thalassemia, an inherited blood disorder | FDA

Capture URL: <https://www.fda.gov/drugs/news-events-human-drugs/fda-approves-first-oral-treatment-anemia-thalassemia-inherited-blood-disorder>

Page loaded at (UTC): Tue, 18 Aug 2026 20:03:49 GMT

Capture timestamp (UTC): Tue, 18 Aug 2026 20:03:52 GMT

Capture tool: 11.2.8

Collection server IP: 54.145.42.72

Browser engine: Mozilla/5.0 (X11; Linux x86_64) AppleWebKit/537.36 (KHTML, like Gecko) @page-vault/browser/11.2.8 Chrome/148.0.7778.280 Safari/537.36

Operating system: Linux (Node 24.18.0)

PDF length: 3

Capture ID: sfoBB9vVFZH2hfCdcfWMXo

Display Name: dani.green