News & Resources

News
- AABB Newsfeed
- AABB News Magazine
- TRANSFUSION Journal

Resources

FDA APPROVES NEW TREATMENT FOR HEMOLYTIC ANEMIA IN ADULTS WITH PK DEFICIENCY

February 18, 2022

The Food and Drug Administration **approved** mitapivat (Pyrukynd, Agios) on Thursday to treat hemolytic anemia in adult patients with pyruvate kinase (PK) deficiency. Mitapivat is the first FDA-approved disease-modifying therapy for PK deficiency, a rare, inherited blood disorder that may cause red cell destruction.

Investigators evaluated the effectiveness of mitapivat in two studies: a randomized, double-blind, placebo-controlled clinical study of 80 adults with PK deficiency who did not receive regular blood transfusions and a single-arm study of 27 adults with PK deficiency who received regular blood transfusions. Participants received 50 mitapivat for an average duration of about 24 weeks in the randomized study and an average duration of about 40 weeks in the single-arm study.

In the randomized study, the effectiveness of mitapivat was based upon hemoglobin response (defined as a 1.5 g/dL or greater increase in hemoglobin concentration that was sustained at two or more scheduled assessments). At the end of the study, 40% of participants in the treatment group had a hemoglobin response, compared with zero participants in the placebo group.

In the single-arm study, effectiveness was based on reduction in transfusion burden (at least a 33% reduction in the number of RBC units transfused during the last 24 weeks of treatment compared with the historical transfusion burden on the individual participant). Thirty-three percent of participants who received mitapivat met this reduction in transfusion burden; 22% of participants did not require any transfusions during the last 24 weeks of treatment.

In the approval notice, FDA noted that abruptly stopping mitapivat could worsen premature red blood cell destruction.

Related News

AABB History: The Formation of a New American Association of Blood Banks
February 17, 2022

After the end of World War II, many professionals recognized the need for a national blood banking organization through which they could share best practices, provide staff training and standardize procedures.

REGULATORY UPDATE: New CBER Web Page Addresses International Efforts to Combat COVID-19
February 17, 2022

The Centers for Biologics Evaluation and Research (CBER) recently introduced a new web page that describes the agency's efforts to address COVID-19 internationally, including regulatory and research collaborations.

REGULATORY UPDATE: FDA Resumes Domestic Inspections
February 9, 2022

The Food and Drug Administration resumed domestic surveillance inspections on Monday in response to the decline in COVID-19 cases across the country.