BlueCross BlueShield
Federal Employee Program

PYRUKYND
(mitapivat)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Pyrukynd (mitapivat) is a pyruvate kinase activator that acts by allosterically binding to the pyruvate kinase tetramer and increasing pyruvate kinase (PK) activity. The red blood cell (RBC) form of pyruvate kinase (PK-R) is mutated in PK deficiency, which leads to reduced adenosine triphosphate (ATP), shortened RBC lifespan, and chronic hemolysis (1).

Regulatory Status

FDA-approved indication: Pyrukynd is a pyruvate kinase activator indicated for the treatment of hemolytic anemia in adults with pyruvate kinase (PK) deficiency (1).

Pyrukynd dosing is driven by hemoglobin levels and transfusion requirements. If no benefit has been observed by 24 weeks, Pyrukynd should be discontinued (1).

Acute hemolysis with subsequent anemia has been observed following abrupt interruption or discontinuation of Pyrukynd. Pyrukynd should not be discontinued abruptly. The dose should be gradually tapered to discontinue treatment if possible. When discontinuing treatment, patients should be monitored for signs of acute hemolysis and anemia including jaundice, scleral icterus, dark urine, dizziness, confusion, fatigue, or shortness of breath (1).

Patients were included in the Pyrukynd clinical trial if they had documented presence of at least 2 variant alleles in the pyruvate kinase liver and red blood cell (PKLR) gene, of which at least 1 was a missense variant, and hemoglobin (Hb) less than or equal to 10 g/dL. Patients who were homozygous for the c.1436G>A (p.R479H) variant or had 2 non-missense variants (without the presence of another missense variant) in the PKLR gene were excluded because these patients did not achieve Hb response (change from baseline in Hb ≥1.5 g/dL at >50% assessments) in the dose-ranging study (1).

The safety and effectiveness of Pyrukynd in pediatric patients less than 18 years of age have not been established (1).

Summary

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Pyrukynd (mitapivat) is indicated for the treatment of hemolytic anemia in adults with pyruvate kinase (PK) deficiency. Pyrukynd dosing is driven by hemoglobin levels and transfusion requirements. If no benefit has been observed by 24 weeks, Pyrukynd should be discontinued. Abrupt interruption or discontinuation should be avoided to minimize the risk of acute hemolysis. The safety and effectiveness of Pyrukynd in pediatric patients less than 18 years of age have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate and cost-effective use of Pyrukynd while maintaining optimal therapeutic outcomes.

References

1. Pyrukynd [package insert]. Cambridge, MA: Agios Pharmaceuticals, Inc.; February 2022.