Drugs

PEER-REVIEWED FEATURE

Mitapivat: Adis Evaluation

Clinical Considerations

- First-in-class PK activator for use in adults with PK deficiency
- Twice-daily oral administration
- Increases haemoglobin levels in adults with PK deficiency who are not regularly RBC transfused
- Reduces RBC transfusion burden in adults with PK deficiency who are regularly RBC transfused
- Generally well tolerated

Plain Language Summary

Background and rationale

- Pyruvate kinase (PK) deficiency is a rare hereditary disease caused by mutations affecting the function of the red blood cell (RBC) PK enzyme
- PK deficiency is characterized by chronic haemolytic anaemia
- Treatment options for adults with PK deficiency have historically been limited to supportive care and are themselves associated with potentially serious complications
- Oral mitapivat (Pyrukynd®) is the first disease-modifying drug to be approved for treatment of PK deficiency in adults in the EU and UK and is approved for the treatment of haemolytic anaemia in adults with PK deficiency in the USA
- Mitapivat acts by restoring activity of the dysfunctional RBC PK enzyme

Clinical findings

- Based on findings from two phase III clinical trials, twice-daily oral mitapivat provided clinical benefit in adults with PK deficiency, both in patients not requiring, and requiring, regular RBC transfusions
- Improvements in disease-specific health-related quality of life were observed in adults with PK deficiency treated with mitapivat
- Mitapivat was generally well tolerated, with most adverse events being mild to moderate in severity

Conclusion

Current evidence indicates that mitapivat is a valuable treatment option for adults with PK deficiency