BlueCross BlueShield Federal Employee Program

FERRIPROX (deferiprone)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Ferriprox (deferiprone) is an iron chelator used to treat patients with iron overload. Ferriprox is a chelating agent with an affinity for ferric ions (ion III) and binds with ferric ions to form neutral 3:1 (deferiprone:iron) complexes that are stable at physiological pH (1).

Regulatory Status

FDA-approved indications: Ferriprox is an iron chelator indicated for: (1)

- the treatment of transfusional iron overload in adult and pediatric patients 8 years of age and older with thalassemia syndromes.
- the treatment of transfusional iron overload in adult and pediatric patients 8 years of age and older with sickle cell disease or other anemias.

Limitations of Use:

Safety and effectiveness have not been established for the treatment of transfusional iron overload in patients with myelodysplastic syndrome or in patients with Diamond Blackfan anemia (1).

Monitor serum ferritin concentration every two to three months to assess the effects of Ferriprox on body iron stores. Dose adjustments should be tailored to the individual patient's response and therapeutic goals (maintenance or reduction of body iron burden). If the serum ferritin falls consistently below 500 mcg/L, consider temporarily interrupting Ferriprox therapy. Monitor serum liver transaminase levels monthly during therapy and consider interrupting treatment if there are consistently elevated transaminase levels (1).

Ferriprox carries a boxed warning regarding agranulocytosis that can lead to serious infections and death. Neutropenia may precede the development of agranulocytosis. Measure the absolute neutrophil count (ANC) before starting Ferriprox therapy and monitor the ANC weekly during therapy. Interrupt Ferriprox therapy if neutropenia develops (ANC $<1.5 \times 10^9$/L). If infection develops, interrupt Ferriprox and monitor the

ANC more frequently. Advise patients taking Ferriprox to report immediately any symptoms indicative of infection (1).

Ferriprox can cause fetal harm when administered to a pregnant woman. Women of reproductive potential should be advised of the potential hazard to the fetus and to avoid pregnancy while on this drug (1).

The safety and effectiveness of Ferriprox tablets in pediatric patients less than 8 years of age have not been established (1).

Summary

Ferriprox (deferiprone) is an iron chelator approved for the treatment of patients with transfusional iron overload. Ferriprox can cause agranulocytosis that can lead to serious infections and death. Neutropenia may precede the development of agranulocytosis. The safety and effectiveness of Ferriprox tablets for oral use in pediatric patients less than 8 years of age have not been established (1).

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

References

1. Ferriprox [package insert]. Toronto, ON: Apotex Inc.; November 2021.