RATIONALE FOR INCLUSION IN PA PROGRAM

Background
Ferriprox is an iron chelator to treat patients with iron overload due to blood transfusions in patients with thalassemia, a genetic blood disorder that causes anemia, who had an inadequate response to prior chelation therapy. Patients with thalassemia have excess iron in the body from the frequent blood transfusions (transfusional iron overload), a condition that is serious and can be fatal. These patients also have a risk of developing liver disease, diabetes, arthritis, heart failure or an abnormal heart rhythm. The standard of care to treat transfusional iron overload is chelation therapy – chemical agents that are used to remove heavy metals from the body. Ferriprox is intended for use when chelation therapy is inadequate (1).

Regulatory Status
FDA-approved indication: Ferriprox is an iron chelator indicated for the treatment of patients with transfusional iron overload due to thalassemia syndromes when current chelation therapy is inadequate (2).

Limitations of Use:
Safety and effectiveness have not been established for the treatment of transfusional iron overload in patients with other chronic anemias (2).

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 (2).

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 x 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 (2).

Ferriprox can cause fetal harm when administered to a pregnant woman. Women of child-bearing age should be advised of the potential hazard to the fetus and to avoid pregnancy while on this drug (2).

The safety and effectiveness of Ferriprox tablets for oral use in pediatric patients have not been established (2).

Summary
Ferriprox is an iron chelator approved for the treatment of patients with transfusional iron overload due to thalassemia syndromes when current chelation therapy is inadequate. Safety and effectiveness have not been established for the treatment of transfusional iron overload in patients with other chronic anemia’s. 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 have not been established (2).

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

References