Ferriprox® (deferiprone) – New indication, expanded indication

- On May 1, 2021, Chiesi Global Rare Diseases announced the FDA approval of Ferriprox (deferiprone), for the treatment of transfusional iron overload in adult and pediatric patients 3 years of age and older with sickle cell disease or other anemias.
    - 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.
- The FDA also expanded Ferriprox's previous indication in transfusional iron overload in thalassemia syndromes to include patients regardless of prior chelation exposure.
    - Ferriprox was previously only approved for this indication when current chelation therapy was inadequate.
- The approval of Ferriprox for the new indication was based on an actively controlled, non-inferiority study comparing Ferriprox to deferoxamine in 185 patients with SCD and other transfusion-dependent anemias by evaluating liver iron concentration (LIC).
    - Over 12 months, the least squares estimate of mean decrease from baseline in LIC was 4.13 ± 0.50 mg/g dry weight (dw) for Ferriprox and 4.38 ± 0.59 mg/g dw for deferoxamine, and the non-inferiority criterion was met.
- Ferriprox carries a boxed warning for agranulocytosis and neutropenia.
- The most common adverse reactions (≥ 6%) with Ferriprox use in patients with SCD or other anemias were pyrexia, abdominal pain, bone pain, headache, vomiting, pain in extremity, sickle cell anemia with crisis, back pain, increased alanine aminotransferase (ALT), increased aspartate aminotransferase (AST), arthralgia, oropharyngeal pain, nasopharyngitis, decreased neutrophil count, cough, and nausea.
- The recommended starting dosage of Ferriprox for all indications is 25 mg/kg (actual body weight) orally, three times per day for a total of 75 mg/kg/day. The dose should be rounded to the nearest 2.5 mL.
    - Dosage adjustments should be tailored to the individual patient's response and therapeutic goals (maintenance or reduction of body iron burden). The maximum oral dosage is 33 mg/kg (actual body weight), three times per day for a total of 99 mg/kg/day.
    - Refer to the Ferriprox drug label for complete dosing and administration recommendations.