Ferriprox® (deferiprone) – New formulation approval

- On May 21, 2020, Chiesi Global Rare Diseases announced the FDA approval of Ferriprox (deferiprone) twice-a-day 1000 mg oral tablets for the treatment of patients with transfusional iron overload due to thalassemia syndromes when current chelation therapy is inadequate.
    - Approval is based on a reduction in serum ferritin levels. There are no controlled trials demonstrating a direct treatment benefit, such as improvement in disease-related symptoms, functioning, or increased survival.
    - Safety and effectiveness have not been established for the treatment of transfusional iron overload in patients with other chronic anemias.
- Ferriprox was previously approved as three times a day tablets.
- The approval for twice-a-day Ferriprox was supported by bioequivalence studies.
- Ferriprox carries a boxed warning for agranulocytosis and neutropenia.
- Additional warnings and precautions for Ferriprox include liver enzyme elevations, zinc deficiency, and embryo-fetal toxicity
- The most common adverse reactions (≥ 5%) with Ferriprox use were nausea, vomiting and abdominal pain, alanine aminotransferase increased, arthralgia and neutropenia.
- The recommended starting dose of the new formulation of Ferriprox is 75 mg/kg/day (actual body weight) in two divided doses. The maximum oral dosage is 99 mg/kg/day (actual body weight) in two divided doses.
- Chiesi launch plans for Ferriprox are pending. Ferriprox will be available as a 1000 mg oral tablet with functional scoring.