Global Rare Diseases

Chiesi Group Receives FDA Approval for Ferriprox® (deferiprone) twice-a-day tablets

BOSTON, May 21, 2020 – Chiesi Global Rare Diseases, a business unit of Chiesi Farmaceutici S.p.A., an international research-focused healthcare Group (Chiesi Group), today announced that the U.S. Food and Drug Administration (FDA) has approved Ferriprox® (deferiprone) twice-a-day tablets for the treatment of patients with transfusional iron overload due to thalassemia syndromes when current chelation therapy is inadequate[1]. The new formulation of twice-a-day Ferriprox 1000 mg oral tablets eliminates the mid-day dose.

"The availability of a new twice-a-day oral tablet formulation of Ferriprox provides patients with the ability to reduce serum ferritin levels and cardiac and liver iron," said Giacomo Chiesi, Head of Chiesi Global Rare Diseases "This important milestone for Chiesi Global Rare Diseases is another reflection of our commitment to innovation to improve the lives of patients living with rare diseases around the world."

Thalassemia syndromes are a group of rare inherited hematological conditions including beta-thalassemia and are characterized by impaired hemoglobin production. In certain cases, such as with beta-thalassemia, severe forms of this genetic disorder if left untreated can lead to life-threatening complications. Patients are often managed with long-term blood transfusions that can put them at risk of developing very high levels of iron in their blood and vital organs. As the level of iron rises, it can generate free radicals that can be toxic to proteins and membranes. Deferiprone is engineered to bind to iron in the tissues and circulation, thereby inactivating it. Iron is then excreted from the body primarily via urine. Dosing is initiated at 75 mg/kg/day and can be increased up to 99 mg/kg/day to improve efficacy in iron chelation. The application for marketing approval was supported by bioequivalence studies.

"In the management of patients with thalassemia, clinicians often see firsthand the difficulties they can have with dosing and compliance, and the impact that this can have on the effectiveness of treatment," said Thomas Coates, M.D. Section Head, Hematology at Children's Hospital Los Angeles. "A treatment option that reduces serum ferritin, cardiac iron and liver iron with an established safety profile and now twice-a-day tablet dosing can represent a significant advantage for patients."

To support patients treated with Ferriprox (twice-a-day), Chiesi Global Rare Diseases offers the Chiesi Total Care™ Program. Through this program, HCPs and patients can access a team of dedicated specialists able to provide individual support as well as information about prescription,

reimbursement, financial assistance and product refills. For information about Chiesi Total Care, call 866-758-7071.

Indication

Ferriprox (twice-a-day) is an iron chelator indicated 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.

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

Important Safety Information

WARNING: AGRANULOCYTOSIS AND NEUTROPENIA

- FERRIPROX can cause 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 and monitor weekly while on therapy.
- Interrupt FERRIPROX if infection develops and monitor the ANC more frequently.
- Advise patients taking FERRIPROX to report immediately any symptoms indicative of infection.

Ferriprox is a contraindicated in patients with known hypersensitivity to deferiprone or to any of the excipients in the formulation.

In clinical studies, 7.5% of 642 patients treated with deferiprone developed increased ALT values. Four (0.62%) deferiprone-treated subjects discontinued the drug due to increase serum ALT levels and 1 (0.16%) due to an increase in both ALT and AST. Monitor serum ALT values monthly during therapy with Ferriprox and consider interruption of therapy if there is a persistent increase in the serum transaminase levels. Decrease plasma zinc concentrations have been observed on deferiprone therapy. Monitor plasma zinc, and supplement in the event of a deficiency.

Ferriprox DR can cause fetal harm. Advise females of reproductive potential to use an effective method of contraception during treatment with Ferriprox and for at least six months after the last dose.

Avoid co-administration of Ferriprox with other drugs known to be associated with neutropenia or agranulocytosis; however, if this is unavoidable, closely monitor the absolute neutrophil count. Allow at least a 4-hour interval between administration of Ferriprox and drugs or supplements containing polyvalent cation (e.g., iron, aluminum, or zinc).

The most common adverse reactions are (incidence ≥5%) nausea, vomiting and abdominal pain, alanine aminotransferase increased, arthralgia, and neutropenia.

Advise patients to avoid alcohol while taking Ferriprox. Consumption of alcohol while taking Ferriprox may result in more rapid release of deferiprone.

Please see full Prescribing Information, including boxed WARNING and Medication Guide.

1. Ferriprox® (deferiprone) Prescribing Information. Chiesi, 2020

About Chiesi Global Rare Diseases
Chiesi Global Rare Diseases is a business unit of the Chiesi Group established in February 2020 and focused on research and development of treatments for rare and ultra-rare disorders. The Global Rare Diseases unit works in collaboration with Chiesi Group to harness the full resources and capabilities of our global network to bring innovative new treatment options to people living with rare diseases, many of whom have limited or no treatments available. The unit is also a dedicated partner with global leaders in patient advocacy, research and patient care.

About Chiesi Group
Based in Parma, Italy, Chiesi Farmaceutici is an international research-focused healthcare group with 85 years of experience in the pharmaceutical industry and a global presence in 29 countries. Chiesi researches, develops, and markets innovative drugs in the respiratory therapeutics, specialist medicine, and rare disease areas. Its R&D organization is headquartered in Parma (Italy), and is integrated with R&D groups in France, the USA, the UK, Canada, and Sweden to advance Chiesi's pre-clinical, clinical, and registration programs. Chiesi employs nearly 6,000 people. Chiesi Group is a certified Benefit corporation. For more information, please visit www.chiesi.com.

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