



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMA/473109/2019  
EMA/H/C/000236

## Ferriprox (*deferiprone*)

An overview of Ferriprox and why it is authorised in the EU

### What is Ferriprox and what is it used for?

Ferriprox is an 'iron chelator' (a substance that attaches to iron) that is used to treat iron overload (an excess of iron in the body) in patients with thalassaemia major. This is an inherited disease in which patients are unable to make enough haemoglobin, the protein found in red blood cells that carries oxygen around the body.

Ferriprox is used:

- on its own, when standard iron chelator treatment cannot be used or does not work well enough;
- in combination with another iron chelator, when treatment with one iron chelator on its own does not work or when iron levels need to be rapidly or intensively corrected to prevent or treat life-threatening conditions (mainly affecting the heart).

Ferriprox contains the active substance deferiprone.

### How is Ferriprox used?

Ferriprox can only be obtained with a prescription and treatment should be started and continued by a doctor who has experience in the treatment of thalassaemia. It is available as tablets (500 and 1,000 mg) and as an oral solution (100 mg/ml).

The usual dose of Ferriprox is 25 mg per kilogram body weight three times a day. The doctor may adjust the dose of Ferriprox according to how well the patient's iron levels are being controlled, which should be measured every 2 to 3 months with blood tests. The total dose for the whole day should be less than 100 mg per kilogram body weight. The doctor may interrupt treatment if iron levels get too low.

For more information about using Ferriprox, see the package leaflet or contact your doctor or pharmacist.

### How does Ferriprox work?

Patients with thalassaemia major need frequent blood transfusions. Transfused red cells bring iron into the body. With repeated transfusions, the iron builds up because the body does not have a natural way

---

**Official address** Domenico Scarlattilaan 6 • 1083 HS Amsterdam • The Netherlands

**Address for visits and deliveries** Refer to [www.ema.europa.eu/how-to-find-us](http://www.ema.europa.eu/how-to-find-us)

**Send us a question** Go to [www.ema.europa.eu/contact](http://www.ema.europa.eu/contact) **Telephone** +31 (0)88 781 6000

An agency of the European Union



of removing excess iron. Over time, the excess iron can damage important organs such as the heart and liver. The active substance in Ferriprox, deferiprone, is an iron chelator. It attaches to iron in the body to form a compound that can be removed from the body, mainly in the urine, and to a lesser extent in the stools. Removing iron in this way helps to correct the iron overload and prevent damage from excess iron.

## **What benefits of Ferriprox have been shown in studies?**

Ferriprox was studied in 247 patients with thalassaemia major, aged over 6 years. The main measure of effectiveness was the change in the levels of ferritin in the blood. The blood ferritin level indicates how much iron is being stored in the body.

The main study involving 71 patients compared Ferriprox with deferoxamine, another iron chelator, over 2 years. Deferoxamine was given by injection under the skin overnight. The average blood ferritin levels were similar in the two treatment groups. However, the average iron concentration in the liver of Ferriprox-treated patients seemed to increase more than in deferoxamine-treated patients.

In another study involving 60 patients treated over 12 months, a combination of Ferriprox and deferoxamine (Ferriprox for 5 days plus deferoxamine for 2 days each week) was compared with deferoxamine on its own. Blood ferritin levels were reduced to the same extent in patients treated with the combination as with deferoxamine on its own. There were too few patients in the study to prove whether such a schedule is as effective as deferoxamine on its own.

In addition, published studies on Ferriprox together with deferoxamine reported greater reductions in blood ferritin levels when both medicines were used in combination compared with using either medicine on its own. In a published study, Ferriprox together with deferoxamine also led to greater decreases in iron in the heart compared with patients taking deferoxamine alone.

## **What are the risks associated with Ferriprox?**

The most common side effects with Ferriprox (which may affect more than 1 in 10 people) are red-brown urine (showing that iron is being removed through the urine), nausea (feeling sick), abdominal pain (stomach ache) and vomiting. Less common but more serious side effects are agranulocytosis (very low levels of granulocytes, a type of white blood cell) and neutropenia (low levels of neutrophils, a type of white blood cell that fights infections).

Ferriprox must not be used in people who have had neutropenia repeatedly or agranulocytosis. Ferriprox must also not be used with medicines that might cause neutropenia or agranulocytosis. When taking Ferriprox, the patient's neutrophil count should be checked regularly (every week in the first year then less frequently). If the patient gets an infection, treatment with Ferriprox should be temporarily stopped and the neutrophil count checked more often. Patients should tell their doctor immediately if they have symptoms of an infection, such as fever, sore throat and flu-like symptoms.

Ferriprox must not be used in women who are pregnant or breastfeeding.

For the full list of restrictions and side effects of Ferriprox, see the package leaflet.

## **Why is Ferriprox authorised in the EU?**

The European Medicines Agency decided that Ferriprox's benefits are greater than its risks and it can be authorised for use in the EU.

## **What measures are being taken to ensure the safe and effective use of Ferriprox?**

The company that markets Ferriprox must supply a reminder card for patients or their carers about the importance of regular tests for neutrophil counts, the need to watch out for symptoms of infection and for women to avoid becoming pregnant during treatment with Ferriprox.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Ferriprox have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Ferriprox are continuously monitored. Side effects reported with Ferriprox are carefully evaluated and any necessary action taken to protect patients.

## **Other information about Ferriprox**

Ferriprox received a marketing authorisation valid throughout the EU on 25 August 1999.

Further information on Ferriprox can be found on the Agency's website:  
[ema.europa.eu/en/medicines/human/EPAR/ferriprox](http://ema.europa.eu/en/medicines/human/EPAR/ferriprox).

This overview was last updated in 09-2019.