



Deferiprone treatment for iron overload

A patient's guide

What is deferiprone?

- Deferiprone is an iron chelation medication.
- Chelation is a small molecule that binds iron and removes it from the body.

Why is too much body iron a bad thing?

- Too much body iron is harmful to the tissues where it accumulates.
- Blood transfusion results in iron overload because each unit of blood contains iron as part of the haemoglobin in red blood cells.
- The body has no natural way of removing all the extra iron so the iron is stored in cells.
- The liver is the main site for iron storage, but once the liver is full of iron the body starts to deposit iron in other organs such as the hormone glands and the heart.
- **Liver complications**
 - Although the liver is better equipped to deal with iron overload than other tissues, poor control of liver iron will cause liver scarring and eventually liver failure (cirrhosis) – although this can take many years to develop.
 - Liver cancer is a late complication.
 - Removing iron from the liver reduces this risk.
- **Hormone problems**
 - Iron can cause serious complications when it is deposited in the hormone glands. If iron is deposited in the pituitary gland, which controls the hormones regulating growth and sexual development, young people may not enter puberty naturally.
 - Older people may develop secondary infertility because the hormones oestrogen (in women) and testosterone (in men) will stop being produced.
 - Other glands that are affected are the thyroid gland (resulting in hypothyroidism), the pancreas (resulting in diabetes), and the parathyroid gland (resulting in low calcium levels in the blood).
 - Endocrine damage is not reversible.
- **Heart problems**
 - Iron can be deposited in the heart and if severe levels are reached can result in heart failure or abnormal palpitations of the heart. These are both serious and can be life threatening.
 - Heart failure is reversible with intensive chelation, but it is completely avoidable if you follow treatment correctly.
- The aim is to prevent problems developing. Therefore, the best strategy for managing iron overload is to avoid high levels of iron.
- In general, we know that liver iron values of above 7mg/g dry weight on a type of MRI called FerriScan MRI (liver iron assessment) are the levels above which complications start to develop.



- We keep a liver iron target of below 5mg/g dry weight for our patients.
- All chelation drugs are effective if used appropriately. We will adjust treatments, so they are tolerable and fit in with your lifestyle, as well as achieving the desired therapeutic goal.

When can Deferiprone be a useful treatment?

- Deferiprone is used to chelate iron in patients with thalassaemia who have been unable to tolerate desferrioxamine or deferasirox, or in patients who need combination therapy.
- It is used outside the remit of its licence in patients with sickle cell disorders and in patients with rare inherited anaemias and more recently in patients with neurodegenerative conditions such as Frederichs ataxia and superficial siderosis.
- You should not take deferiprone if:
 - You are pregnant, trying for a baby or breast-feeding
 - You have ever been told you have a low number of white blood cells
 - You have previously had a low white cell count on deferiprone.

How does Deferiprone work?

- Deferiprone is small enough to be absorbed through the gut and enter the blood stream.
- Binding of iron can occur in the blood stream or within tissues such as the liver or heart.
- Once iron is bound to deferiprone, the iron complex is eliminated from the body through the urine. The urine turns a reddish colour.

How is Deferiprone taken?

- Deferiprone is available as 500mg tablets, 1,000mg tablets and an oral solution.
- It is taken orally as three divided doses a day.
- Allow at least a four-hour interval between deferiprone and other medications or supplements containing aluminium or zinc.

How much should be taken and how often?

- Deferiprone is taken orally and can be taken with or without food.
- If you miss a dose, take it as soon as you remember. If it is almost time for your next dose, skip the missed dose and then continue with your regular schedule. Do not try to catch up or take two doses at the same time to make up for a missed dose.
- If the goal is to maintain a safe level of iron then we will normally use a dose of around 75mg/kg/day.
- We may use doses of up to 100mg/kg/day if the goal is to reduce the total iron burden or if someone is on a very heavy transfusion programme.
- We may also recommend that deferiprone is used with desferrioxamine infusions in a combination regime. This combination can be used in patients with iron in the heart or in patients with high levels of liver iron.
- As the iron burden comes down, the doses will be reduced so that we can maintain the iron at a safe level.



Can deferiprone be given with other iron chelators?

- Deferiprone is often used as a single chelation treatment or as part of a combination treatment with desferrioxamine or deferasirox.

Monitoring for effectiveness of deferiprone

- As with other chelators, the serum ferritin levels and trend is the most convenient way to monitor iron overload.
- An MRI scan of the liver and heart can be used for dose and regime adjustment and for risk assessment. These are typically performed about once a year.

How frequent are unwanted effects and what are they?

Like any medicine, deferiprone can have unwanted effects.

- **Agranulocytosis or neutropenia**
 - Deferiprone can cause a serious side effect resulting in a very low white blood cell count.
 - One type of white blood cell, called a neutrophil, is important for fighting bacterial infections.
 - If you have a low neutrophil count (called neutropenia) you may be at risk of developing serious infection.
 - Neutropenia is quite common with deferiprone and around 4% of patients may have a low neutrophil count occasionally.
 - If the low neutrophil count continues to fall, it can become a serious problem.
 - Severe neutropenia is known as agranulocytosis (1% of patients may get this) and if you develop it, you will be at risk of having serious infections that can lead to death unless promptly identified and appropriately treated.
 - It is very important that you have **regular blood tests** to check the neutrophil count while taking deferiprone.
 - If you develop neutropenia, your healthcare professional should check your blood counts every day until your white blood cell count improves.
 - **Should you develop a fever or a sore throat while taking deferiprone please do not take the next dose and attend hospital urgently for a full blood count to exclude neutropenia or agranulocytosis.**
 - If you are unwell you will need to be admitted to hospital and will require antibiotics and sometimes growth factor injections to help bring the neutrophil count back up.
- **Effects on the gut**
 - These occur in about 15% to 30% of patients, are typically mild and do not persist.
 - These include stomach pain (often described as hunger cramps), nausea, and vomiting.
 - These symptoms rarely require dose adjustment or stopping treatment and tend to settle down over a few weeks.
 - If these persist, or you suffer from severe vomiting or nausea every time you take deferiprone, the medical team prescribing the treatment should be informed.
 - Mostly these symptoms can be managed effectively by taking the deferiprone with food or by starting at a low dose and increasing the dose every few days until you are on the correct dose.
 - Occasionally you may need anti-nausea medication to help you.



- **Effects on the liver**

- Deferiprone can cause increased liver enzymes in your blood.
- Your medical team should do a monthly blood test to check your liver function during treatment.
- Mostly these are mild increases, and no change is needed but if there is a large increase in the value of the liver enzymes, the medical team may stop the deferiprone and consider investigations.

- **Joint pains**

- These can occur in about 15% of patients and are mostly mild or moderate.
- These tend to occur in large joints such as the knee or ankle.
- If you get such pains or if the joint becomes swollen, let your medical team know.
- These side effects can occur even after many years on deferiprone.

- **Red urine**

- This is a very common side effect, and most patients will get reddish coloured urine after taking deferiprone.
- This is the iron to drug complex and reflects the impact of chelation.

Monitoring for side effects of deferiprone

- Monitoring is important to assess for side effects. You should have the following tests done on a regular basis:
 1. Full blood count regularly for monitoring the neutrophils
 2. Liver function tests once a month to look for liver problems with deferiprone
 3. All the routine monitoring for iron overload including ferritin tests, MRI assessments of liver and heart iron as scheduled by the medical team.

Contact details

- Your consultant team is:

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- Adult departmental contacts:

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- Network website:
<https://www.uclh.nhs.uk/theredcellnetwork/subscribe>

If you or your family have any other questions, please do not hesitate to contact any of the above medical team at Whittington Health.

Where can I get more information?

The UK Thalassaemia Society

19 The Broadway, London N14 6PH

Tel: 020 8882 0011

Website: www.ukts.org

The Sickle Cell Society

54 Station Rd, London NW10 4UA

Tel: 020 8861 7795

Website: <http://www.sicklecellsociety.org>

NHS 111

Tel: 111

www.nhs.uk/NHSEngland/AboutNHSservices/Emergencyandurgentcareservices/Pages/NHS-111.aspx

Patient advice and liaison service (PALS)

If you have a compliment, complaint or concern please contact our PALS team on 020 7288 5551 or whh-tr.PALS@nhs.net

If you need a large print, audio or translated copy of this leaflet please email whh-tr.patient-information@nhs.net. We will try our best to meet your needs.

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