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Fabhalta (*iptacopan*)

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

What is Fabhalta and what is it used for?

Fabhalta is a medicine used in adults with paroxysmal nocturnal haemoglobinuria (PNH) to treat haemolytic anaemia.

PNH is a disease in which the excessive breakdown of blood cells results in anaemia (low levels of haemoglobin, the protein in red blood cells that carries oxygen around the body), thrombosis (blood clots in blood vessels), pancytopenia (low levels of blood cells) and dark urine (due to large amounts of haemoglobin being released into the urine).

PNH is rare, and Fabhalta was designated an 'orphan medicine' (a medicine used in rare diseases) on 4 June 2020. Further information on the orphan designation can be found on the EMA website.

Fabhalta contains the active substance iptacopan.

How is Fabhalta used?

Fabhalta is available as capsules to be taken by mouth twice a day. If one or more doses are missed, the medicine should be taken as soon as possible. If multiple doses are missed, patients should be monitored for signs and symptoms of haemolysis.

The medicine can only be obtained with a prescription.

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

How does Fabhalta work?

The complement system is a set of proteins that is part of the immune system (the body's natural defences). In patients with PNH, the complement system is over-active and damages the patients' own blood cells.

The active substance in Fabhalta, iptacopan, blocks a protein of the complement system called 'factor B'. By blocking factor B, Fabhalta prevents the complement system from damaging cells, especially red blood cells, thereby helping to relieve the symptoms of the disease.

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
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

What benefits of Fabhalta have been shown in studies?

Fabhalta was shown to be effective at increasing haemoglobin levels and reducing the need for blood transfusions in one main study involving 97 patients with PNH.

Patients in the study had been previously treated with ravulizumab or eculizumab (other medicines for PNH) for at least 6 months and still had anaemia. Patients took either Fabhalta or continued their treatment with ravulizumab or eculizumab.

After 24 weeks of treatment, the percentage of patients who achieved an increase in haemoglobin levels of at least 2 g/dL without blood transfusions was around 82% for patients on Fabhalta , compared with 2% of patients continuing on ravulizumab or eculizumab. Around 69% of patients taking Fabhalta achieved haemoglobin levels of at least 12 g/dL without blood transfusions, compared with around 2% of the patients taking ravulizumab or eculizumab.

Data from an additional study supported the use of Fabhalta in patients with PNH who had not been previously treated.

What are the risks associated with Fabhalta?

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

The most common side effects with Fabhalta (which may affect more than 1 in 10 people) include upper respiratory tract (nose and throat) infection, headache and diarrhoea. In the clinical studies, the most common serious side effect was urinary tract infection.

Based on how Fabhalta works, it may increase the risk of infections. Fabhalta must not be used by patients who have an ongoing infection caused by so-called encapsulated bacteria, including *Neisseria meningitidis*, *Streptococcus pneumoniae* and *Haemophilus influenzae* type B. It must also not be used by patients who are not currently vaccinated against *N. meningitidis* and *S. pneumoniae* unless the risk of delaying treatment outweighs the risk of developing an infection from these bacteria.

Why is Fabhalta authorised in the EU?

Fabhalta was shown to be effective at increasing haemoglobin levels and reducing the need for blood transfusions in patients with PNH. The most common side effects are considered inconvenient but are not expected to pose a risk to patients. The European Medicines Agency therefore decided that Fabhalta's benefits are greater than its risks and that it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Fabhalta?

The company that markets Fabhalta will provide doctor and patients with educational material on the risk of infections caused by encapsulated bacteria and of serious haemolysis following treatment termination.

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

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

Other information about Fabhalta

Fabhalta received a marketing authorisation valid throughout the EU on 17 May 2024.

Further information on Fabhalta can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/fabhalta

This overview was last updated in 05-2024.