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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 to treat:

- haemolytic anaemia in adults with paroxysmal nocturnal haemoglobinuria (PNH). 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).
- complement 3 glomerulopathy (C3G) in adults, either in combination with a RAS inhibitor (a medicine that acts on the renin-angiotensin system) or alone in patients who cannot take a RAS inhibitor. C3G is a disease in which progressive damage to the kidneys prevent them from filtering the blood correctly, leading to toxin buildup, reduced urine production and swelling.

PNH and C3G are rare, and Fabhalta was designated an 'orphan medicine' (a medicine used in rare diseases). Further information on the orphan designations can be found on the EMA website (PNH: 4 June 2020; C3G: 4 December 2018).

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. In patients with PNH, if multiple doses are missed, patients should be monitored for signs and symptoms of haemolysis (an abnormal breakdown of red blood cells).

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.

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How does Fabhalta work?

The complement system is a group of proteins that is part of the immune system (the body's natural defences). In patients with PNH and C3G, the complement system is over-active, damaging the patients' own cells, particularly red blood cells in PNH and kidney cells in C3G.

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 red blood cells in PNH and kidney cells in C3G, thereby helping to relieve the symptoms of these diseases.

What benefits of Fabhalta have been shown in studies?

PNH

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.

C3G

Fabhalta was shown to be more effective than placebo at reducing kidney damage in one main study involving 74 patients with C3G. Patients in the study were already treated for C3G with other medicines such as RAS inhibitors and immunosuppressants (corticosteroids, mycophenolate mofetil or mycophenolate sodium).

The main measure of effectiveness was protein levels in the urine of patients. As healthy kidneys keep nearly all proteins in the blood, the presence of proteins in urine indicates kidney damage.

After 6 months of treatment, patients given Fabhalta had an approximately 35% reduction in their urine protein levels compared with those given placebo. This effect was maintained after 12 months of treatment.

What are the risks associated with Fabhalta?

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

In patients with PNH, 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. The most common serious side effect with Fabhalta is urinary tract infection (infection of the parts of the body that collect and pass out urine), which may affect up to 1 in 10 patients.

In patients with C3G, the most common side effects with Fabhalta (which may affect more than 1 in 10 people) include upper respiratory tract infection. The most common serious side effect is infection with pneumococcal bacteria, which may affect up to 1 in 10 people.

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. It was also shown to reduce the accumulation of proteins in the urine of patients with C3G, which suggests reduced kidney damage. 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 doctors and patients with educational materials on the risk of infection by encapsulated bacteria and the need for patients to receive appropriate vaccination. For patients with PNH, the materials also include information on the risk of serious haemolysis when stopping treatment with Fabhalta.

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 03-2025.