

Summary report on authorisation dated 28 February 2025

## Fabhalta<sup>®</sup> (active substance: iptacopan)

Authorisation in Switzerland: 29 August 2024

Hard capsules as monotherapy for the treatment of adults with paroxysmal nocturnal haemoglobinuria (PNH)

### About the medicinal product

Fabhalta contains the active substance iptacopan (as iptacopan hydrochloride monohydrate).

Fabhalta is used to treat paroxysmal nocturnal haemoglobinuria (PNH) in adults who have an inadequate response to treatment lasting at least 6 months with another type of medicinal product for PNH, known as a C5 inhibitor. In this case, Fabhalta is used as monotherapy (i.e. as the only medication).

In patients with PNH, the body's own defence system (complement system) is

overactive and attacks the red blood cells. This can result in anaemia, fatigue, functional impairments, stomach pain, dark urine, shortness of breath, difficulty swallowing, erectile dysfunction, and the formation of blood clots.

Since PNH is a rare, severe, and potentially life-threatening disease, the medicinal product Fabhalta has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

### Mode of action

Iptacopan, the active substance in Fabhalta, binds to a specific protein called Factor B and

blocks the complement system, which attacks the red blood cells. Fabhalta increases the number of red blood cells (reduces the anaemia) and controls the PNH.

### Use

Fabhalta is a prescription-only medicine.

It is available in the form of hard capsules, with each capsule containing 200 mg of iptacopan (as iptacopan hydrochloride monohydrate).

The recommended dosage is 200 mg (1 capsule) twice daily (total dosage of 400 mg daily).

The capsules should be swallowed whole and can be taken with or without food.

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## Efficacy

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The efficacy of Fabhalta was investigated in a study (APPLY-PNH) with 97 PNH patients who had an inadequate response to treatment lasting at least 6 months with a C5 inhibitor.

The patients were divided into 2 groups: one group received 200 mg Fabhalta twice daily and the other group continued C5 inhibitor treatment.

After 24 weeks, treatment with Fabhalta demonstrated a sustained increase in haemoglobin values<sup>1</sup> by  $\geq 2$  g/dL compared to baseline in 82.3% of cases and stable haemoglobin values of  $\geq 12$  g/dL in 68.8% of cases, without a blood transfusion being necessary. These results were significantly better compared to the control group, who did not receive Fabhalta.

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## Precautions, undesirable effects, & risks

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Fabhalta must not be used in those who are hypersensitive to the active substance or any of the excipients.

The most common undesirable effects (affecting more than 1 in 10 patients) are upper respiratory tract infections (25.5%), headache (18.1%), abdominal pain (12.8%), and diarrhoea (10.1%).

As there is a risk of severe infections, patients taking Fabhalta should be vaccinated against bacteria such as *Neisseria meningitidis* and *Streptococcus pneumoniae*.

All precautions, risks, and other possible undesirable effects are listed in the Information for patients (package leaflet) and the Information for healthcare professionals.

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## Why the medicinal product has been authorised

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There are currently limited treatment options for patients with paroxysmal nocturnal haemoglobinuria (PNH) who still suffer from haemolysis despite treatment with C5 inhibitors. In the study described, Fabhalta demonstrated a significant improvement in haemoglobin values and a reduction in the need for transfusions in these patients.

Taking all the risks and precautions into account, and based on the available data, the benefits of Fabhalta outweigh the risks.

Swissmedic has therefore authorised the medicinal product Fabhalta, containing the active substance iptacopan, in Switzerland for the treatment of paroxysmal nocturnal haemoglobinuria (PNH) in adults who have an inadequate response to treatment lasting at least 6 months with a C5 inhibitor.

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## Further information on the medicinal product

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Information for healthcare professionals: [Information for healthcare professionals Fabhalta®](#)

Information for patients (package leaflet): [Information for patients Fabhalta®](#)

Healthcare professionals can answer any further questions.

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<sup>1</sup> Haemoglobin value: Haemoglobin is a protein in red blood cells that is responsible for the transport of oxygen in the blood. The haemoglobin value shows how much haemoglo-

bin is present in the blood and is measured in grams per decilitre (g/dL). A normal haemoglobin value in adults is between 12 and 16 g/dL for women and between 14 and 18 g/dL for men.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Summary report on authorisation.

Swissmedic monitors medicinal products authorised in Switzerland. Swissmedic initiates the necessary action in the event of newly discovered adverse drug reactions or other safety-relevant signals. New findings that could impair the quality, efficacy, or safety of this medicinal product are recorded and published by Swissmedic. If necessary, the medicinal product information is adapted.