21 March 2024
EMA/CHMP/54481/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Fabhalta

iptacopan

On 21 March 2024, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Fabhalta[2], intended for the treatment of paroxysmal nocturnal haemoglobinuria (PNH). The applicant for this medicinal product is Novartis Europharm Limited.

Fabhalta will be available as a 200 mg hard capsule. The active substance of Fabhalta is iptacopan, a complement inhibitor (ATC code: L04AJ08). Iptacopan targets Factor B to selectively inhibit the alternative complement pathway and control both C3-mediated extravascular haemolysis and terminal complement-mediated intravascular haemolysis.

The benefit of Fabhalta is its ability to prevent haemolysis to increase and maintain haemoglobin levels in patients with PNH, as shown in both an active comparator-controlled and single-arm phase 3 study. The most common side effects are upper respiratory tract infection, headache and diarrhoea.

The full indication is:

> Fabhalta is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia.

Fabhalta should be prescribed by physicians experienced in the management of patients with haematological disorders.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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