# Healthcare professional's guide to FABHALTA®▼ (iptacopan)

This brochure has been developed by Novartis Ireland Ltd. to fulfil the conditions of the marketing authorisation and is intended for Healthcare Professionals prescribing FABHALTA® (iptacopan). It should be read along with the Summary of Product Characteristics (SmPC), available at www.medicines.ie.

▼This medicinal product is subject to additional monitoring. Reporting suspected adverse reactions after authorisation of the medicinal product is important. It allows continued monitoring of the benefit/risk profile of the medicinal product. All suspected adverse reactions should be reported to HPRA Pharmacovigilance at www.hpra.ie. Adverse events can also be reported to Novartis preferably at www.novartis.com/report, by emailing drugsafety.dublin@novartis.com or by calling (01) 2080 612.

If there are any questions or concerns about iptacopan, speak with a Novartis representative.

FABHALTA®▼
iptacopan

IE11351172 | October 2025
Version 1.0
Approved by HPRA November 2025

# Introduction

Iptacopan is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia.[1]

This guide provides healthcare professionals with safety information and guidance on the serious risks associated with the use of iptacopan.

**Briefly, you should:**

- Be aware of the risks of infection and haemolysis
- Ensure that your patient has received the appropriate vaccinations or antibiotic prophylaxis for encapsulated bacteria and is revaccinated as recommended

## Important note

Iptacopan is provided under controlled access as part of its Risk Management Plan (RMP) and can only be dispensed on receipt of written confirmation that a patient has received the appropriate vaccinations (including Neisseria meningitidis, Streptococcus pneumoniae and, if appropriate, Haemophilus influenzae type B) or antibiotic prophylaxis. The HSE Managed Access Protocol form for iptacopan will contain a section where Healthcare Professionals must attest that the patient has received the necessary vaccinations prior to treatment. A prescription cannot be ordered or dispensed by a pharmacy until such evidence is provided on the relevant managed access application and approved by the HSE Medicines Management Programme.

If you prescribe iptacopan you will receive an annual reminder of mandatory re-vaccinations in line with current national vaccination guidelines including Neisseria meningitidis, Streptococcus pneumoniae and, if appropriate, Haemophilus influenzae type B).

As part of the RMP, patients receiving iptacopan must be given the following materials to support their treatment:

- **Patient and caregiver guide**, to inform patients and caregivers about the potential risks associated with iptacopan treatment and their mitigation
- **Patient safety card**, containing key safety information for patients and healthcare professionals about the risk of serious infections with iptacopan, including contact details for the patient's prescriber

Please advise your patients to carry their patient safety card with them at all times during their treatment, and for 2 weeks following their last iptacopan dose, in case of an emergency.



# What is in this guide?

# Risk of serious infections[1]

**Iptacopan may increase the risk of serious, life-threatening or fatal infections caused by encapsulated bacteria,** including *Neisseria meningitidis, Streptococcus pneumoniae, and Haemophilus influenzae* Type B.

### During treatment with iptacopan

Monitor patients for signs and symptoms of sepsis, meningitis or pneumonia, such as:

- **Fever**
  - With or without shivers or chills
  - With a headache
  - With a rash
  - With chest pain and cough
  - With breathlessness/ fast breathing
  - With high heart rate
- **Headache**
  - With nausea or vomiting
  - With a stiff neck or stiff back
- **Confusion**
- **Body aches with flu-like symptoms**
- **Clammy skin**
- **Eyes sensitive to light**

**If bacterial infection is suspected, treat with antibiotics immediately.**



# Prophylactic vaccinations and/or antibiotic treatment[1]

### Before starting treatment with iptacopan

Ensure patients are vaccinated against *Neisseria meningitidis* and *Streptococcus pneumoniae* according to the current national vaccination guidelines before treatment with iptacopan. The *Haemophilus influenzae* Type B vaccine is recommended for patients where it is available.

Patients must be vaccinated against encapsulated bacteria at least 2 weeks before starting treatment with iptacopan, and/or receive antibiotic prophylaxis until 2 weeks after vaccination.

If immediate treatment with iptacopan is required, administer the necessary vaccines as soon as possible. Additionally, appropriate prophylactic antibiotics should be given to the patient until 2 weeks after vaccination. This should be in accordance with current national recommendations.

Re-vaccinate when necessary, according to current national vaccination guidelines.

Carefully monitor patients for early signs of serious infections as the measures above reduce, but do not eliminate, the risk of developing an infection. Inform patients and carefully monitor them for signs and symptoms of infection. Treat any suspected infections immediately.

# Risk of serious haemolysis after discontinuing iptacopan[1]

**Discontinuation of iptacopan may increase the risk of serious haemolysis.**

This means it is important to provide patients and their caregivers with advice on adherence to the dosing schedule. Patients are at risk of serious haemolysis for at least 2 weeks after discontinuing treatment with iptacopan. Closely monitor patients for signs and symptoms during this period.

Possible signs and symptoms of haemolysis include, but are not limited to:

- **Elevated lactate dehydrogenase (LDH) levels along with sudden decrease in haemoglobin or PNH clone size**
- **Fatigue**
- **Haemoglobinuria**
- **Abdominal pain**
- **Dyspnoea**
- **Dysphagia**
- **Erectile dysfunction**
- **Major adverse vascular events, including venous or arterial thrombosis**

If iptacopan treatment must be discontinued, alternative therapy should be considered.

If haemolysis occurs after discontinuation of iptacopan, consider restarting treatment with iptacopan.



# The Post-Authorisation Safety Study (PASS)

Novartis is conducting a global PASS that aims to characterise the identified and potential risks of iptacopan in routine clinical practice. Further study objectives are to provide additional data on use in pregnancy and long-term safety, and to evaluate effectiveness of the measures related to the required and recommended vaccinations in the iptacopan-treated PNH population.

The PASS aims to use the data collected through the International PNH Interest Group (IPIG) Registry. The aim of the registry is to develop an international database to prospectively collect observational data on PNH (regardless of treatment received). The PASS will cover patient characteristics, clinical outcomes, pregnancy outcomes as well as long-term safety data on patients treated with iptacopan in selected registry sites.

Inform patients about the PASS. If your clinical site participates in the iptacopan PASS data collection, they will automatically be enrolled if they consented to the collection of their clinical data via the IPIG PNH Registry.

If you have questions about the PASS, please contact your local Novartis office via medinfo.dublin@novartis.com.

IPIG, International PNH Interest Group; LDH, lactate dehydrogenase; PASS, Post-Authorisation Safety Study; PNH, paroxysmal nocturnal haemoglobinuria; RMP, Risk Management Plan.

Reference: 1. FABHALTA® (iptacopan) Summary of Product Characteristics, Available at www.medicines.ie

IE11351172 | October 2025
Approved by HPRA November 2025
FABHALTA® EU RMP HCP Guide, version 1.0


NOVARTIS