Medicines Management Programme

Managed Access Protocol – Iptacopan (FABHALTA®) as monotherapy in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH)

Medicine	Date of addition to Managed Access Protocol
Iptacopan (FABHALTA®)	01/12/2025

Approved by	**Professor Michael Barry, Clinical Lead, MMP**	
Date approved	Version 1.0	20/11/2025

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List of abbreviations

C3	Component 3
C5	Component 5
EVH	Extravascular haemolysis
HSE	Health Service Executive
HTH	High Tech Hub
IVH	Intravascular haemolysis
MAP	Managed Access Protocol
Mg	Milligram
MMP	Medicines Management Programme
PCRS	Primary Care Reimbursement Service
PIGA	Phosphatidylinositol glycan anchor biosynthesis class A
PNH	Paroxysmal nocturnal haemoglobinuria
SmPC	Summary of product characteristics

1. Iptacopan

FABHALTA® contains iptacopan. Iptacopan is an inhibitor of Factor B in the alternative pathway of the complement cascade. It prevents the activation of component 3 (C3) convertase and the subsequent formation of component 5 (C5) convertase to control both C3 mediated extravascular haemolysis (EVH) and terminal complement-mediated intravascular haemolysis (IVH).

From December 2025, one presentation of iptacopan is available on the High Tech Arrangement.

- FABHALTA® 200 mg hard capsules

1.1 Licensed indication(s)

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

In addition, iptacopan (FABHALTA®) is indicated in other disease areas that are outside the scope of this managed access protocol (MAP).

1.2 Reimbursement

Reimbursement of iptacopan on the High Tech Arrangement for the treatment of PNH is supported for adult patients with PNH who have haemolytic anaemia and who meet the criteria outlined in this MAP. All criteria must be satisfied in order for reimbursement to be supported.

An application for reimbursement approval is required to be submitted on an individual patient basis. The *Iptacopan (FABHALTA®) Reimbursement Application Form* should be completed and sent by secure email to the Health Service Executive (HSE)-Medicines Management Programme (MMP) at mmp@hse.ie.

Table 1 outlines the licensed therapeutic dosage of iptacopan for the treatment of PNH in adult patients who have haemolytic anaemia. Please refer to the Summary of Product Characteristics (SmPC) for further prescribing information.

Table 1 Licensed therapeutic dosage of iptacopan

Medicine	Route of administration	Dosage
Iptacopan	Oral	200 mg twice daily

mg: milligrams

If a patient is recommended for reimbursement of iptacopan, reimbursement is supported up to the maximum licensed dosage specified in Table 1. Reimbursement of dosages in excess of the licensed therapeutic dosage (as outlined in Table 1) is not supported.

Reimbursement is not supported for long-term concomitant use of complement inhibitors for PNH.

See Section 3 for further details on Reimbursement criteria – Requirement for outcome data.

1.3 Reimbursement price

The reimbursement price of the presentation of iptacopan available on the High Tech Arrangement is outlined in Table 2. Commercial-in-confidence arrangements are in place with the marketing authorisation holder to reduce the net acquisition cost of iptacopan to the HSE.

Table 2 Reimbursement code and price for the presentation of iptacopan available on the High Tech Arrangement

Strength (pack size)	Code	Reimbursement price*
FABHALTA® 200 mg capsule (56 capsules)	89469	€31,050.00

mg: milligrams
*Correct as at 01 December 2025

2. Reimbursement criteria – Initiation

This section outlines the criteria that must be satisfied in order for patients to be recommended for reimbursement of iptacopan on the High Tech Arrangement.

2.1 Prescribers

Applications for reimbursement approval for iptacopan for the treatment of PNH on the High Tech Arrangement will only be considered from consultants with specialist registration in haematology with the Irish Medical Council and who have agreed to the terms of this MAP and been approved by the HSE ('approved consultants').

Approved consultants are responsible for ensuring that the patient or their representative/guardian is aware that the application for reimbursement approval is being made on their behalf.

The prescribing of FABHALTA® for approved patients for the treatment of PNH on the High Tech Arrangement will be confined to the approved consultants and their teams. The governance of the team on the High Tech Hub, including access, rests with the approved consultant.

2.2 Patient age

Applications for reimbursement approval will only be considered for individuals aged 18 years or older at time of application.

2.3 Patient diagnosis

For a positive reimbursement recommendation, approved consultants will be required to confirm a diagnosis of PNH at the time of application. The items outlined below (2.3.1 – 2.3.6) are required to confirm the diagnosis of PNH and to exclude other potential differential diagnoses. This is not an exhaustive list of requirements; the HSE-MMP reserve the right to request additional information as deemed necessary to enable a reimbursement recommendation.

2.3.1 Flow cytometry

The approved consultant will be required to provide a flow cytometry report that shows evidence of CD59 and CD55 negative cells.

2.3.2 Full blood count and haemolytic blood panel

The approved consultant will be required to provide a full blood count and a haemolytic blood panel to support the diagnosis of PNH and confirm the presence of haemolysis and anaemia.

2.3.3 Biochemistry profile

The approved consultant will be required to provide a biochemistry profile to support the diagnosis of PNH.

2.3.4 Bone marrow investigations

The approved consultant will be required to provide bone marrow investigations (bone marrow aspirate and/or biopsy) to support the diagnosis or exclude a differential diagnosis.

2.3.5 Genetic analysis

The approved consultant may be required to provide a genetic report, confirming a phosphatidylinositol glycan anchor biosynthesis class A (PIGA)-gene mutation, to aid in the diagnosis, if available.

2.3.6 Other symptoms of PNH

The approved consultant will be required to provide details of other presenting symptoms of PNH including e.g. abdominal pain, dyspnoea, fatigue, jaundice, thrombosis.

2.4 Patient clinical history/status

In line with the exclusion criteria from the clinical trials and SmPC for FABHALTA®, applications for reimbursement approval will not be considered for individuals who:

- meet any of the contraindications for treatment as outlined in the FABHALTA® SmPC
- have a diagnosis of hereditary complement deficiency.

2.4.1 Thrombosis history

The approved consultant will be required to confirm whether the patient has a history of, or is at risk of thrombosis.

2.4.2 Haemolytic anaemia

The approved consultant will be required to confirm that the patient is experiencing haemolytic anaemia.

2.4.3 Mandatory vaccinations and antibiotics

The approved consultant will be required to confirm that the patient will be vaccinated against Neisseria meningitidis and Streptococcus pneumoniae infections and/or will receive prophylactic antibiotics.

2.5 Patient's medical treatment

2.5.1 Blood transfusion history

The approved consultant will be required to provide details of the patient's past and current requirement for blood transfusions.

2.5.2 Patient medication history

The approved consultant will be required to provide a complete list of the patient's current drug therapy at the time of application or any relevant previous treatments.

3.Reimbursement criteria – Requirement for outcome data

Follow-up data may be requested by the MMP for audit purposes and provision of same is a condition of ongoing reimbursement. It is the responsibility of the approved consultant to ensure

that the patient or their representative/guardian is aware that the provision of follow-up data is a condition of reimbursement, and that audits may occur during which their personal data will be reviewed.

4.Prescribing of iptacopan for approved patients

Please refer to the SmPC for FABHALTA® for full prescribing information including monitoring and patient counselling requirements.

If a patient is recommended for reimbursement by the HSE-MMP, the High Tech prescription should be generated on the High Tech Hub (HTH). High Tech prescriptions which are not hub generated for iptacopan will not be eligible for reimbursement by the HSE-Primary Care Reimbursement Service (PCRS). Only approved consultants and their teams will have access to generate prescriptions.

HSE-Managed Access Protocol Declaration for Approved Consultants for iptacopan (FABHALTA®) for PNH in adult patients with haemolytic anaemia

I, the undersigned, agree to seek reimbursement approval for, and prescribe this medicine, iptacopan (FABHALTA®), as per the managed access protocol as outlined above.	
Name of consultant	
Irish Medical Council registration number	
Signature of consultant	
Institution	
Date	
For internal use: *Approval status and date approved*	