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Section:	Prescription Drugs	**Effective Date:**	July 1, 2025
Subsection:	Hematological Agents	**Original Policy Date:**	December 29, 2023
Subject:	Fabhalta	**Page:**	1 of 7

Last Review Date: June 12, 2025

Fabhalta

Description

Fabhalta (iptacopan)

Background
Fabhalta (iptacopan) binds to Factor B of the alternative complement pathway and regulates the cleavage of C3, generation of downstream effectors, and the amplification of the terminal pathway. In paroxysmal nocturnal hemoglobinuria (PNH), intravascular hemolysis (IVH) is mediated by the downstream membrane attack complex, while extravascular hemolysis (EVH) is facilitated by C3b opsonization. Fabhalta acts proximally in the alternative pathway of the complement cascade to control both Cb3-mediated EVH and terminal complement-mediated IVH. In primary immunoglobulin A nephropathy (IgAN), the deposition of galactose deficient IgA1 containing immune complexes in the kidney locally activates the alternative complement pathway which is thought to contribute to the pathogenesis of IgAN. In 3 glomerulopathy (C3G), overactivation of the alternative complement pathway leads to C3 cleavage within the glomeruli resulting in C3 deposition and inflammation, which are thought to contribute to the pathogenesis of C3G (1).

Regulatory Status
FDA-approved indications: Fabhalta is a complement factor B inhibitor, indicated for: (1)

- the treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH).
- the reduction of proteinuria in adults with primary immunoglobulin A nephropathy (IgAN) at risk of rapid disease progression, generally a urine protein-to-creatinine ratio (UPCR) ≥1.5 g/g.

- the treatment of adults with complement 3 glomerulopathy (C3G), to reduce proteinuria.

Fabhalta has a boxed warning regarding serious infections caused by encapsulated bacteria. Fabhalta increases the risk of serious infections, especially those caused by encapsulated bacteria, such as *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type B. These infections may become rapidly life-threatening or fatal if not recognized and treated early. Patients should be vaccinated against encapsulated bacteria at least 2 weeks prior to initiation of Fabhalta therapy according to current Advisory Committee on Immunization Practices (ACIP) guidelines. Patients should be monitored for early signs and symptoms of serious infections and evaluate immediately if infection is suspected. Because of the risk of serious infections caused by encapsulated bacteria, Fabhalta is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the Fabhalta REMS (1).

Fabhalta also has warnings regarding hyperlipidemia and monitoring of PNH manifestations after Fabhalta discontinuation (1).

The safety and effectiveness of Fabhalta in pediatric patients less than 18 years of age have not been established (1).

Related policies
Empaveli, Filspari, Soliris, Tarpeyo, Ultomiris

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Fabhalta may be considered **medically necessary** if the conditions indicated below are met.

Fabhalta may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 18 years of age or older

Diagnosis

Patient must have the following:

1. Paroxysmal nocturnal hemoglobinuria (PNH)

AND ALL of the following:
a. Documented baseline value for hemoglobin (Hgb)
b. Vaccination against encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type B at least 2 weeks prior to initiation [unless Fabhalta (iptacopan) treatment cannot be delayed]
c. Prescriber is enrolled in the Fabhalta REMS program
d. **NO** dual therapy with another Prior Authorization (PA) medication for PNH (see Appendix 1)

Age 18 years of age or older

Diagnosis

Patient must have the following:

1. Primary immunoglobulin A nephropathy (IgAN)

AND ALL of the following:
a. Diagnosis has been confirmed by a kidney biopsy
b. Patient is at risk of rapid disease progression indicated by a urine protein-to-creatinine ratio (UPCR) ≥1.5 g/g
c. Used in combination with maximum recommended or maximum tolerated dose of ACEI or ARB therapy
d. eGFR ≥ 20 mL/min/1.73 m2
e. Prescribed by or recommended by a nephrologist
f. Vaccination against encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type B at least 2 weeks prior to initiation [unless Fabhalta (iptacopan) treatment cannot be delayed]
e. Prescriber is enrolled in the Fabhalta REMS program

Age 18 years of age or older

Diagnosis

Patient must have the following:

1. Complement 3 glomerulopathy (C3G)

AND ALL of the following:

a. Diagnosis has been confirmed by a kidney biopsy
b. Used to reduce proteinuria
c. Documented baseline urine protein-to-creatinine ratio (UPCR)
d. Used in combination with maximum recommended or maximum tolerated dose of ACEI or ARB therapy
e. Vaccination against encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type B at least 2 weeks prior to initiation [unless Fabhalta (iptacopan) treatment cannot be delayed]
f. Prescriber is enrolled in the Fabhalta REMS program

Prior – Approval *Renewal* Requirements

Age 18 years of age or older

Diagnosis

Patient must have the following:

1. Paroxysmal nocturnal hemoglobinuria (PNH)

AND ALL of the following:

a. Increase in hemoglobin (Hgb) from pretreatment baseline
b. Absence of unacceptable toxicity from the drug
c. Prescriber is enrolled in the Fabhalta REMS program
d. **NO** dual therapy with another Prior Authorization (PA) medication for PNH (see Appendix 1)

Age 18 years of age or older

Diagnoses

Patient must have **ONE** of the following:

1. Primary immunoglobulin A nephropathy (IgAN)

2. Complement 3 glomerulopathy (C3G)

AND ALL of the following:

a. Decrease in urine protein-to-creatinine ratio (UPCR)
b. Prescriber is enrolled in the Fabhalta REMS program
c. Used in combination with maximum recommended or maximum tolerated dose of ACEI or ARB therapy

Policy Guidelines

Pre – PA Allowance

None

Prior - Approval Limits

Quantity 400 mg per day

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary

Fabhalta is a complement factor B inhibitor indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH), primary immunoglobin A nephropathy (IgAN), and complement 3 glomerulopathy (C3G). Fabhalta has a boxed warning citing the risk of serious infections caused by encapsulated bacteria and it is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). The safety and effectiveness of Fabhalta in pediatric patients less than 18 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Fabhalta while maintaining optimal therapeutic outcomes.

References

1. Fabhalta [package insert]. East Hanover, NJ: Novartis Pharmaceuticals Corporation; March 2025.

Policy History

Date	Action
December 2023	Addition to PA
March 2024	Annual review
June 2024	Annual review and reference update
September 2024	Annual review
October 2024	Per PI update, added indication of primary immunoglobulin A nephropathy (IgAN)
December 2024	Annual review
March 2025	Annual review
April 2025	Per PI update, added indication of complement 3 glomerulopathy (C3G)
June 2025	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 12, 2025 and is effective on July 1, 2025.

Appendix 1 - List of PA Medications for PNH

Generic Name	Brand Name
eculizumab	Soliris
iptacopan	Fabhalta
pegcetacoplan	Empaveli
ravulizumab-cwvz	Ultomiris