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Section:	Prescription Drugs	**Effective Date:**	April 1, 2024
Subsection:	Hematological Agents	**Original Policy Date:**	December 29, 2023
Subject:	Fabhalta	**Page:**	1 of 5

Last Review Date: March 8, 2024

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-meidated EVH and terminal complement-mediated IVH (1).

Regulatory Status
FDA-approved indication: Fabhalta is a complement factor B inhibitor, indicated for the treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH) (1).

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, Soliris, 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 Fabhalta REMS program
d. **NO** dual therapy with another terminal complement inhibitor (see Appendix 1)

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 Fabhalta REMS program
d. **NO** dual therapy with another terminal complement inhibitor (see Appendix 1)

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). 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; December 2023.

Policy History

Date	Action
December 2023	Addition to PA
March 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 8, 2024 and is effective on April 1, 2024.

Appendix 1 - List of Terminal Complement Inhibitors

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