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# 5.85.055

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**Last Review Date:** March 7, 2025

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## Fabhalta

### Description

#### Fabhalta (iptacopan)

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#### 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 C3-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 (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)  $\geq 1.5$  g/g.

Fabhalta has a boxed warning regarding serious infections caused by encapsulated bacteria. Fabhalta increases the risk of serious infections, especially those caused by encapsulated

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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).

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## 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:

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- 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)
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**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)  $\geq 1.5$  g/g
  - c. Used in combination with maximum recommended or maximum tolerated dose of ACEI or ARB therapy
  - d. eGFR  $\geq 20$  mL/min/1.73 m<sup>2</sup>
  - 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]
  - g. Prescriber is enrolled in the Fabhalta REMS program
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## Prior – Approval *Renewal* Requirements

**Age** 18 years of age or older

## Diagnosis

Patient must have the following:

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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)

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**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. 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

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### Prior – Approval *Renewal* Limits

Same as above

## Rationale

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## Summary

Fabhalta is a complement factor B inhibitor indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH) and primary immunoglobulin A nephropathy (IgAN). 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; August 2024.

## 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

## Keywords

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**This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.**

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## Appendix 1 - List of PA Medications for PNH

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