

DRUG POLICY

Fabhalta® (iptacopan)

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy. For this program, Epysqli (eculizumab-aagh) and Ultomiris (ravulizumab) are the preferred products and will apply to members requesting treatment for paroxysmal nocturnal hemoglobinuria (PNH). The criteria will require the use of one of the health plan's preferred products for PNH before the use of non-preferred products (Soliris, Bkembv, Empaveli, and Fabhalta) unless there are clinical circumstances that exclude the use of all the preferred products.

FDA-Approved Indications

Fabhalta (iptacopan) is indicated for:

1. The treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH)
2. To reduce 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*.
3. Treatment of adults with complement 3 glomerulopathy (C3G), to reduce proteinuria.

*This indication is approved under accelerated approval based on reduction of proteinuria. It has not been established whether Fabhalta slows kidney function decline in patients with IgAN. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory clinical trial.

POLICY

Required Documentation

Submission of the following information is necessary to initiate the prior authorization review:

1. For initial requests:

- A. Paroxysmal nocturnal hemoglobinuria: Flow cytometry used to show results of glycosylphosphatidylinositol-anchored proteins (GPI-APs) deficiency
 - B. Primary immunoglobulin A nephropathy (IgAN):
 - i. Kidney biopsy confirming a diagnosis of primary immunoglobulin A nephropathy (IgAN).
 - ii. Laboratory report and/or chart note(s) indicating the member is at risk for disease progression with a UPCR greater than or equal to 1.5 g/g.
 - C. Complement 3 glomerulopathy (C3G);
 - i. Kidney biopsy confirming a diagnosis of complement 3 glomerulopathy (C3G).
 - ii. Laboratory report and/or chart note(s) indicating the member has proteinuria greater than or equal to 1 g/day or baseline UPCR greater than or equal to 1 g/g.
 - iii. Laboratory report and/or chart note(s) showing a reduction in serum C3.
2. For continuation requests:
- A. Paroxysmal nocturnal hemoglobinuria: Chart notes or medical documentation supporting positive clinical response
 - B. Primary immunoglobulin A nephropathy (IgAN): Laboratory report and/or chart note(s) supporting positive clinical response
 - C. Complement 3 glomerulopathy (C3G): Laboratory report and/or chart notes indicating the member has decreased levels of proteinuria or UPCR from baseline.

Preferred Drug Plan Design

Criteria for initial approval for Fabhalta (iptacopan) for paroxysmal nocturnal hemoglobinuria (PNH) will only apply when one of the following criteria are met:

- 1. The patient has had an inadequate response to treatment, intolerable adverse event, or has a contraindication to therapy with one of the preferred products, Epysqli (eculizumab-aagh) or Ultomiris (ravulizumab).

Prescriber Specialties

The requested medication must be prescribed by or in consultation with one of the following:

- A. Paroxysmal nocturnal hemoglobinuria: hematologist
- B. Primary immunoglobulin A nephropathy and complement 3 glomerulopathy (C3G): nephrologist

Criteria for Initial Approval

Paroxysmal Nocturnal Hemoglobinuria (PNH)

Fabhalta (iptacopan) may be considered **medically necessary** for the treatment of PNH when all of the following criteria are met:

- 1. The diagnosis of PNH was confirmed by detecting a deficiency of glycosylphosphatidylinositol-anchored proteins (GPI-APs) as demonstrated by either of the following:
 - A. At least 5% PNH cells
 - B. At least 51% of GPI-AP deficient poly-morphonuclear cells
- 2. Flow cytometry is used to demonstrate GPI-APs deficiency
- 3. Member has and exhibits clinical manifestations of disease (e.g., LDH > 1.5 ULN, thrombosis, renal dysfunction, pulmonary hypertension, dysphagia).
- 4. Member will not use concomitantly with another complement inhibitor (e.g., Empaveli, Piasky, eculizumab, Ultomiris) for the treatment of PNH.

Approval will be for up to 6 months

Primary immunoglobulin A nephropathy (IgAN)

Fabhalta (iptacopan) may be considered **medically necessary** for the treatment of IgAN when all of the following criteria are met:

1. Member has a diagnosis of primary immunoglobulin A nephropathy (IgAN) confirmed by kidney biopsy.
2. Member is 18 years of age or older
3. Member is at risk for rapid disease progression defined by UPCR greater than or equal to 1.5 g/g
4. Member has received a stable dose of maximally tolerated renin-angiotensin system (RAS) inhibitor therapy (e.g., angiotensin converting enzyme inhibitor [ACEI] or angiotensin II receptor blocker [ARB]) for at least 3 months prior to initiation of therapy, or member has an intolerance or contraindication to RAS inhibitors.
5. Member has tried and failed Filspari unless member has an intolerance or contraindication.
6. The requested medication will not be used in combination with Tarpeyo (budesonide delayed release), Vanrafia (atrasentan), Voyxact (sibeprenlimab-szsi), or Filspari (sparsentan).

Approval will be for up to 12 months

Complement 3 glomerulopathy (C3G)

Fabhalta (iptacopan) may be considered **medically necessary** for the treatment of C3G when all of the following criteria are met:

1. Member has a diagnosis of complement 3 glomerulopathy (C3G) confirmed by kidney biopsy.
2. Member has proteinuria greater than or equal to 1 g/day or UPCR greater than or equal to 1 g/g.
3. Member has reduced serum C3 (defined as less than 0.85 times the lower limit of normal per the reference ranges provided) at baseline.
4. Member has received a stable dose of maximally tolerated renin-angiotensin system (RAS) inhibitor therapy (e.g., angiotensin converting enzyme inhibitor [ACEI] or angiotensin II receptor blocker [ARB]) for at least 3 months prior to initiation of therapy, or member has an intolerance or contraindication to RAS inhibitors.

Approval will be for up to 12 months

Continuation of Therapy

Paroxysmal Nocturnal Hemoglobinuria (PNH)

Fabhalta (iptacopan) may be considered medically necessary for the continued treatment of PNH in members when all of the following is met:

1. There is no evidence of unacceptable toxicity or disease progression while on the current regimen.
2. The member demonstrates a positive response to therapy (e.g., improvement in hemoglobin levels, normalization of lactate dehydrogenase [LDH] levels).
3. The requested medication will not be used in combination with another complement inhibitor (e.g., Empaveli, Piasky, eculizumab, Ultomiris) for the treatment of PNH.

Approval will be for up to 12 months

Primary immunoglobulin A nephropathy (IgAN)

Fabhalta (iptacopan) may be considered medically necessary for the continued treatment of IgAN in members when all of the following is met:

1. There is no evidence of unacceptable toxicity or disease progression while on the current regimen.
2. The member is experiencing benefit from therapy as evidenced by either of the following:
 - i. Decreased levels of proteinuria from baseline.
 - ii. Decrease in UPCR from baseline.

Approval will be for up to 12 months

Complement 3 glomerulopathy (C3G)

Fabhalta (iptacopan) may be considered medically necessary for the continued treatment of C3G in members when all of the following is met:

1. There is no evidence of unacceptable toxicity or disease progression while on the current regimen.
2. The member is experiencing benefit from therapy as evidenced by either of the following:
 - i. Decreased levels of proteinuria from baseline.
 - ii. Decrease in UPCR from baseline.

Approval will be for up to **12 months**

Other

Fabhalta (iptacopan) is considered **not medically necessary** for members who do not meet the criteria set forth above.

Members currently receiving the requested medication as samples or via the manufacturer's patient assistance program will be required to meet the criteria for initial approval. This ensures that members are treated equally regardless of their provider's ability to access medication samples.

Non-Formulary Exception Criteria

Non-Formulary Exception criteria applies to formularies which do not include the requested product(s) on the formulary drug list. Meeting the criteria above may satisfy some, or all, portions of the Non-Formulary Exception Criteria. A medication that is non-formulary may be covered when the Criteria for Approval AND the following criteria are met:

1. The requested drug must be used for an FDA-approved indication, or an indication supported in the compendia of current literature (examples: AHFS, Micromedex, current accepted guidelines). Diagnostic testing/lab results required when applicable.
2. The prescribed dose/quantity must fall within the FDA-approved labeling or dosing guidelines found in the compendia of current literature.
3. All covered formulary alternative drugs on any tier will be ineffective, have been ineffective, would not be as effective as the non-formulary drug, or would have adverse effects. Documentation is required and must include chart note(s) or other documentation indicating prior treatment failure, severity of the adverse event (if any), and dosage and duration of the prior treatment, or contraindication to formulary alternatives.

Dosing and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

Quantity Limits Apply

Medication	Standard Limit	FDA Recommended Dosing
Fabhalta (iptacopan) 200 mg oral capsule	60 capsules per 30 days	200 mg twice daily

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

REFERENCES

- Fabhalta [package insert]. East Hanover, NJ: Novartis Pharmaceuticals Corporation; March 2025.
- Parker CJ. Management of paroxysmal nocturnal hemoglobinuria in the era of complement inhibitory therapy. *Hematology*. 2011; 21-29.
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- Preis M, Lowrey CH. Laboratory tests for paroxysmal nocturnal hemoglobinuria (PNH). *Am J Hematol*. 2014;89(3):339-341.
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- Kidney Disease: Improving Global Outcomes (KDIGO) Glomerular Diseases Work Group. KDIGO 2021 Clinical Practice Guideline for the Management of Glomerular Diseases. *Kidney Int*. 2021 Oct; 100 (4S): S1-S276. doi: 10.1016/j.kint.2021.05.021.

POLICY HISTORY

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