Reference number(s)
6288-A

Specialty Guideline Management
Fabhalta

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Fabhalta	iptacopan

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

Fabhalta is indicated for:

- Treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH).
- 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.
    - 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.
- Treatment of adults with complement 3 glomerulopathy (C3G), to reduce proteinuria.

All other indications are considered experimental/investigational and not medically necessary.

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Documentation

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

For initial requests:

- Paroxysmal nocturnal hemoglobinuria (PNH): Flow cytometry used to show results of glycosylphosphatidylinositol-anchored proteins (GPI-APs) deficiency.
- Primary immunoglobin A nephropathy (IgAN):
  - Kidney biopsy confirming a diagnosis of primary immunoglobulin A nephropathy (IgAN).
  - Laboratory report and/or chart note(s) indicating the member has proteinuria greater than or equal to 0.5 g/day or baseline UPCR greater than or equal to 0.8 g/g obtained within 3 months prior to initiation of the requested drug.
- Complement 3 glomerulopathy (C3G):
  - Kidney biopsy confirming a diagnosis of complement 3 glomerulopathy (C3G).
  - 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 1.0 g/g obtained within 3 months prior to initiation of the requested drug.
  - Laboratory report and/or chart note(s) showing a reduction in serum C3.

For continuation requests:

- Paroxysmal nocturnal hemoglobinuria (PNH): Chart notes or medical record documentation supporting positive clinical response.
- Primary immunoglobin A nephropathy (IgAN): Laboratory report and/or chart note(s) indicating the member has decreased levels of proteinuria or UPCR from baseline.
- Complement 3 glomerulopathy (C3G): Laboratory report and/or chart note(s) indicating the member has decreased levels of proteinuria or UPCR from baseline.

Prescriber Specialties

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

- Primary immunoglobin A nephropathy (IgAN) and complement 3 glomerulopathy (C3G): nephrologist.

Coverage Criteria

Paroxysmal Nocturnal Hemoglobinuria (PNH)[1-6]

Authorization of 6 months may be granted for treatment of paroxysmal nocturnal hemoglobinuria (PNH) when all of the following criteria are met:

- The diagnosis of PNH was confirmed by detecting a deficiency of glycosylphosphatidylinositol-anchored proteins (GPI-APs) (e.g., at least 5% PNH cells, at least 51% of GPI-AP deficient poly-morphonuclear cells).
- Flow cytometry is used to demonstrate GPI-APs deficiency.
- Member has and exhibits clinical manifestations of disease (e.g., lactate dehydrogenase [LDH] > 1.5 upper limit of normal [ULN], thrombosis, renal dysfunction, pulmonary hypertension, dysphagia).
- The requested medication will not be used in combination with another complement inhibitor (e.g., Empaveli, Piasky, Soliris, Ultomiris) for the treatment of PNH.

Primary Immunoglobulin A Nephropathy (IgAN)[1,8]

Authorization of 12 months may be granted for treatment of primary immunoglobulin A nephropathy (IgAN) when all of the following criteria are met:

- Member has a diagnosis of primary immunoglobulin A nephropathy (IgAN) confirmed by kidney biopsy.
- Member has either of the following obtained within 3 months prior to initiation of the requested drug:
    - Proteinuria greater than or equal to 0.5 g/day.
    - UPCR greater than or equal to 0.8 g/g.
- 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.

Complement 3 Glomerulopathy (C3G)[1,7]

Authorization of 12 months may be granted for treatment of complement 3 glomerulopathy (C3G) when all of the following criteria are met:

- Member has a diagnosis of complement 3 glomerulopathy (C3G) confirmed by kidney biopsy.
- Member has either of the following obtained within 3 months prior to initiation of the requested drug:
    - Proteinuria greater than or equal to 1 g/day.
    - UPCR greater than or equal to 1.0 g/g.
- Member has reduced serum C3 (defined as less than 0.85 times the lower limit of normal per the reference ranges provided) at baseline.
- 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.

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Continuation of Therapy

Paroxysmal Nocturnal Hemoglobinuria (PNH)

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization when all of the following criteria are met:

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

Primary Immunoglobulin A Nephropathy (IgAN)

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization when all of the following criteria are met:

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

Complement 3 Glomerulopathy (C3G)

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization when all of the following criteria are met:

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

References

1. Fabhalta [package insert]. East Hanover, NJ: Novartis Pharmaceuticals Corporation; March 2025.
2. Parker CJ. Management of paroxysmal nocturnal hemoglobinuria in the era of complement inhibitory therapy. Hematology. 2011; 21-29.

3. Borowitz MJ, Craig F, DiGiuseppe JA, et al. Guidelines for the Diagnosis and Monitoring of Paroxysmal Nocturnal Hemoglobinuria and Related Disorders by Flow Cytometry. Cytometry B Clin Cytom. 2010: 78: 211-230.
4. Preis M, Lowrey CH. Laboratory tests for paroxysmal nocturnal hemoglobinuria (PNH). Am J Hematol. 2014;89(3):339-341.
5. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemoglobinuria. Hematology Am Soc Hematol Educ Program. 2016;2016(1):208-216.
6. Dezern AE, Borowitz MJ. ICCS/ESCCA consensus guidelines to detect GPI-deficient cells in paroxysmal nocturnal hemoglobinuria (PNH) and related disorders part 1 - clinical utility. Cytometry B Clin Cytom. 2018 Jan;94(1):16-22.
7. 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.
8. Kidney Disease: Improving Global Outcomes (KDIGO). KDIGO 2025 Clinical Practice Guideline for the Management of Immnoglobulin A Nephropathy (IgAN) and Immunoglobulin A Vasculitis (IgAV). Kidney Int. 2025 Oct; 108 (4S): S1-S71.