MEDICAL MUTUAL®

Drug Policy

Policy:	**Fabhalta (**iptacopan**)**	**Annual Review Date:** **09/19/2024** **Last Revised Date:** **09/19/2024**

OVERVIEW

Fabhalta, a Factor B inhibitor, is indicated for the following uses:[1]

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

Fabhalta has a Boxed Warning about serious meningococcal infections.[1] Fabhalta is only available through a restricted access program, Fabhalta Risk Evaluation and Mitigation Strategy (REMS).

POLICY STATEMENT

This policy involves the use of Fabhalta. Prior authorization is recommended for pharmacy benefit coverage of Fabhalta. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Fabhalta as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Fabhalta be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Fabhalta is recommended in those who meet the following criteria:

1. **Paroxysmal Nocturnal Hemoglobinuria.** Approve if the patient meets ONE of the following (A or B):
   - **A)** Initial therapy. Approve for 6 months if the patient meets ALL of the following (i, ii, iii, iv, v, and vi):
     - **i.** Patient is ≥ 18 years of age; AND
     - **ii.** Paroxysmal nocturnal hemoglobinuria diagnosis was confirmed by peripheral blood flow cytometry results showing the absence or deficiency of glycosylphosphatidylinositol-anchored proteins on at least two cell lineages; AND
     - **iii.** Patient has a mean hemoglobin level <10 g/dL; AND

iv. Patient has an LDH level of 1.5 times the upper limit of the normal range; AND
v. Patient has complete or updated vaccinations for encapsulated bacteria (Streptococcus pneumoniae, Neisseria meningitidis, and Haemophilus influenzae) at least 2 weeks prior to the first dose of Fabhalta; AND
vi. The medication is prescribed by or in consultation with a hematologist.

B) Patient is Currently Receiving Fabhalta. Approve if the patient meets the following (i, ii, and iii):
i. Patient is ≥ 18 years of age; AND
ii. Patient is continuing to derive benefit from Fabhalta according to the prescriber; AND
Note: Examples of benefit include increase in or stabilization of hemoglobin levels, decreased transfusion requirements or transfusion independence, reductions in hemolysis.
iii. The medication is prescribed by or in consultation with a hematologist.

2. Primary Immunoglobulin A Nephropathy. Approve for the duration noted if the patient meets ONE of the following (A or B):

1. Initial Therapy. Approve for 9 months if the patient meets ALL of the following (i, ii, iii, iv, v, vi, and vii):
i. Patient is ≥ 18 years of age; AND
ii. The diagnosis has been confirmed by biopsy; AND
iii. Patient is at high risk of disease progression, defined by meeting BOTH of the following (a and b):
a) Patient meets ONE of the following [(1) or (2)]:
1) Proteinuria > 1.0 g/day; OR
1) Urine protein-to-creatinine ratio ≥ 1.5 g/g; AND
a) Patient has received the maximum or maximally tolerated dose of ONE of the following for ≥ 12 weeks prior to starting Fabhalta [(1) or (2)]:
1) Angiotensin converting enzyme inhibitor; OR
1) Angiotensin receptor blocker; AND
iv. Patient has received ≥ 3 months of optimized supportive care, including blood pressure management, lifestyle modification, and cardiovascular risk modification, according to the prescriber; AND
v. Patient has an estimated glomerular filtration rate ≥ 30 mL/min/1.73 m2; AND
vi. Patient has complete or updated vaccinations for encapsulated bacteria (Streptococcus pneumoniae, Neisseria meningitidis, and Haemophilus influenzae) at least 2 weeks prior to the first dose of Fabhalta; AND
vii. The medication is prescribed by or on consultation with a nephrologist.

B) Patient is Currently Receiving Fabhalta. Approve for 1 year if the patient meets ALL of the following (i, ii, iii, iv, and v):
i. Patient is ≥ 18 years of age; AND
ii. The diagnosis has been confirmed by biopsy; AND
iii. According to the prescriber, patient has had a response to Fabhalta; AND
Note: Examples of a response are a reduction in urine protein-to-creatinine ratio from baseline, reduction in proteinuria from baseline.
iv. Patient has an estimated glomerular filtration rate ≥ 30 mL/min/1.73 m^2; AND
v. The medication is prescribed by or on consultation with a nephrologist.

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Initial Approval/ Extended Approval.

A) *Initial Approval:* *6 months*
B) *Extended Approval:* *1 year*

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Fabhalta has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. **Concomitant Use with Another Complement Inhibitor**. There is no evidence to support concomitant use of Fabhalta with another another complement inhibitor.
   Note: Examples of complement inhibitors are Empaveli (pegcetacoplan subcutaneous injection), PiaSky (crovalimab-akkz intravenous infusion or subcutaneous injection), Soliris (eculizumab intravenous infusion), Ultomiris (ravulizumab-cwzy intravenous infusion or subcutaneous injection), and Voydeya (danicopan tablets).

2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational, or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

1. Fabhalta® capsules [prescribing information]. East Hanover, NJ: Novartis; December 2023.
2. Cançado RD, da Silva Araújo A, Sandes AF, et al. Consensus statement for diagnosis and treatment of paroxysmal nocturnal haemoglobinuria. *Hematol Transfus Cell Ther*. 2021;43:341-348.
3. Shah N, Bhatt H. Paroxysmal Nocturnal Hemoglobinuria. [Updated 2023 Jul 31]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2023 Jan. Available from: https://www.ncbi.nlm.nih.gov/books/NBK562292/. Accessed December 18, 2023.

4. Roth A, Maciejewski J, Nishinura JI, et al. Screening and diagnostic clinical algorithm for paroxysmal nocturnal hemoglobinuria: Expert consensus. *Eur J Haematol*. 2018;101(1):3-11.