

Drug Policy: Fabhalta™ (iptacopan)

POLICY NUMBER UM ONC_1501	SUBJECT Fabhalta™ (iptacopan)		DEPT/PROGRAM UM Dept	PAGE 1 OF 3
DATES COMMITTEE REVIEWED 06/12/24	APPROVAL DATE June 12, 2024	EFFECTIVE DATE June 28, 2024	COMMITTEE APPROVAL DATES 06/12/24	
PRIMARY BUSINESS OWNER: UM		COMMITTEE/BOARD APPROVAL Utilization Management Committee		
NCQA STANDARDS UM 2		ADDITIONAL AREAS OF IMPACT		
CMS REQUIREMENTS	STATE/FEDERAL REQUIREMENTS		APPLICABLE LINES OF BUSINESS Commercial, Exchange, Medicaid	

I. PURPOSE

To define and describe the accepted indications for Fabhalta (iptacopan) usage in the treatment of cancer, including FDA approved indications, and off-label indications.

Evolent is responsible for processing all medication requests from network ordering providers. Medications not authorized by Evolent may be deemed as not approvable and therefore not reimbursable.

The use of this drug must be supported by one of the following: FDA approved product labeling, CMS-approved compendia, National Comprehensive Cancer Network (NCCN), American Society of Clinical Oncology (ASCO) clinical guidelines, or peer-reviewed literature that meets the requirements of the CMS Medicare Benefit Policy Manual Chapter 15.

II. INDICATIONS FOR USE/INCLUSION CRITERIA

A. Continuation requests for a not-approvable medication shall be exempt from this Evolent policy provided:

1. The member has not experienced disease progression on the requested medication **AND**
2. The requested medication was used within the last year without a lapse of more than 30 days of having an active authorization **AND**
3. Additional medication(s) are not being added to the continuation request.

B. Paroxysmal Nocturnal Hemoglobinuria

1. Fabhalta (iptacopan) may be used for the treatment of adult members with paroxysmal nocturnal hemoglobinuria (PNH).

III. EXCLUSION CRITERIA

- A. Disease progression while taking Fabhalta (iptacopan).

- B. Concurrent use with other therapies used to treat PNH, i.e. eculizumab, ravulizumab.
- C. Dosing exceeds single dose limit of 200 mg.
- D. Investigational use of Fabhalta (iptacopan) with an off-label indication that is not sufficient in evidence or is not generally accepted by the medical community. Sufficient evidence that is not supported by CMS recognized compendia or acceptable peer reviewed literature is defined as any of the following:
 1. Whether the clinical characteristics of the patient and the cancer are adequately represented in the published evidence.
 2. Whether the administered chemotherapy/biologic therapy/immune therapy/targeted therapy/other oncologic therapy regimen is adequately represented in the published evidence.
 3. Whether the reported study outcomes represent clinically meaningful outcomes experienced by patients. Generally, the definitions of Clinically Meaningful outcomes are those recommended by ASCO, e.g., Hazard Ratio of < 0.80 and the recommended survival benefit for OS and PFS should be at least 3 months.
 4. Whether the experimental design, considering the drugs and conditions under investigation, is appropriate to address the investigative question. (For example, in some clinical studies, it may be unnecessary or not feasible to use randomization, double blind trials, placebos, or crossover).
 5. That non-randomized clinical trials with a significant number of subjects may be a basis for supportive clinical evidence for determining accepted uses of drugs.
 6. That case reports are generally considered uncontrolled and anecdotal information and do not provide adequate supportive clinical evidence for determining accepted uses of drugs.
 7. That abstracts (including meeting abstracts) without the full article from the approved peer-reviewed journals lack supporting clinical evidence for determining accepted uses of drugs.

IV. MEDICATION MANAGEMENT

- A. Please refer to the FDA label/package insert for details regarding these topics.

V. APPROVAL AUTHORITY

- A. Review – Utilization Management Department
- B. Final Approval – Utilization Management Committee

VI. ATTACHMENTS

- A. None

VII. REFERENCES

- A. Fabhalta prescribing information 2023. Novartis Pharmaceuticals Corporation East Hanover, New Jersey 07936
- B. Peffault de Latour R, et al. Oral Iptacopan Monotherapy in Paroxysmal Nocturnal Hemoglobinuria. *N Engl J Med.* 2024 Mar 14;390(11):994-1008. doi: 10.1056/NEJMoa2308695
- C. Risitano AM, Kulasekararaj A, Roth A, et al. Factor B inhibition with oral iptacopan monotherapy demonstrates sustained long-term efficacy and safety in anti-C5-treated patients (pts) with paroxysmal nocturnal hemoglobinuria (PNH) and persistent anemia: final 48-week results from the

multicenter, phase III APPLY-PNH trial. Presented at the 2023 American Society of Hematology (ASH) Annual Meeting and Exposition; December 9-12; San Diego, CA; abstract 571.

- D. Jang JH, et al. Iptacopan monotherapy in patients with paroxysmal nocturnal hemoglobinuria: a 2-cohort open-label proof-of-concept study. *Blood Adv.* 2022 Aug 9;6(15):4450-4460. doi: 10.1182/bloodadvances.2022006960.
- E. *Clinical Pharmacology Elsevier Gold Standard 2024.*
- F. *Micromedex® Healthcare Series: Micromedex Drugdex Ann Arbor, Michigan 2024.*
- G. *National Comprehensive Cancer Network. Cancer Guidelines and Drugs and Biologics Compendium 2024.*
- H. Ellis LM, et al. American Society of Clinical Oncology perspective: Raising the bar for clinical trials by defining clinically meaningful outcomes. *J Clin Oncol.* 2014 Apr 20;32(12):1277-80.
- I. Medicare Benefit Policy Manual Chapter 15 Covered Medical and Other Health Services: <https://www.cms.gov/Regulations-and-Guidance/Guidance/Manuals/Downloads/bp102c15.pdf>.