CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Fabhalta (iptacopan)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Fabhalta, approved by the FDA in 2023, is a first-in-class, oral complement factor B inhibitor, indicated for the treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH). Like Empaveli, Fabhalta controls both intravascular and extravascular hemolysis, unlike Soliris and Ultomiris, which only impact intravascular hemolysis. The APPLY-PNH study demonstrated superiority of Fabhalta versus continuation of Soliris or Ultomiris for outcomes including hemoglobin levels and transfusion avoidance.
PNH is a hematopoietic stem cell disorder in which activation of the complement system destroys red blood cells because of an acquired mutation in the *PIGA* gene. Common manifestations can include hemolytic anemia and fatigue. Thrombosis and bone marrow suppression may also occur.

Fabhalta (iptacopan) will be considered for coverage when the following criteria are met:

Paroxysmal Nocturnal Hemoglobinuria (PNH)

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a hematologist; AND
3. Member has a documented diagnosis of PNH as confirmed by high-sensitivity flow cytometry with clone size ≥ 10%; AND
4. Member has a lactate dehydrogenase (LDH) level >1.5x upper limit of normal (ULN); AND
5. Member has at least one PNH-related sign/symptom e.g., fatigue, hemoglobin <10 g/dL, thrombosis, pRBC transfusion, shortness of breath; AND
6. Member has been vaccinated against encapsulated bacteria (Streptococcus pneumoniae, Neisseria meningitidis types A, C, W, Y, and B, and Haemophilus influenzae type B).
7. **Dosage allowed/Quantity limit:** 200 mg orally twice daily. QL: 60 capsules per 30 days.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Chart notes must show clinical evidence of positive response to therapy such as increased hemoglobin level, decreased need for transfusions, normalized LDH levels, improved fatigue.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Fabhalta (iptacopan) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
12/11/2023	New policy for Fabhalta created.

References:

1. Fabhalta [prescribing information]. Novartis Pharmaceuticals Corporation; 2023.
2. Jang JH, Wong L, Ko BS, et al. Iptacopan monotherapy in patients with paroxysmal nocturnal hemoglobinuria: a 2-cohort open-label proof-of-concept study. *Blood Adv*. 2022;6(15):4450-4460. doi:10.1182/bloodadvances.2022006960
3. Ristiano A, Kulasekararaj A, Roeth 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. *Blood* 2023; 142 (Supplement 1): 571.
4. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemoglobinuria. Hematology Am Soc Hematol Educ Program. 2016;2016(1):208-216. doi:10.1182/asheducation-2016.1.208
5. Patriquin CJ, Kiss T, Caplan S, et al. How we treat paroxysmal nocturnal hemoglobinuria: A consensus statement of the Canadian PNH Network and review of the national registry. Eur J Haematol. 2019;102(1):36-52. doi:10.1111/ejh.13176
6. Devos T, Meers S, Boeckx N, et al. Diagnosis and management of PNH: Review and recommendations from a Belgian expert panel. Eur J Haematol. 2018;101(6):737-749. doi:10.1111/ejh.13166
7. Bodó I, Amine I, Boban A, et al. Complement Inhibition in Paroxysmal Nocturnal Hemoglobinuria (PNH): A Systematic Review and Expert Opinion from Central Europe on Special Patient Populations. *Adv Ther*. 2023;40(6):2752-2772. doi:10.1007/s12325-023-02510-4
8. Sahin F, Akay OM, Ayer M, et al. Pesg PNH diagnosis, follow-up and treatment guidelines. *Am J Blood Res*. 2016;6(2):19-27. Published 2016 Aug 5.
9. Cançado RD, Araújo ADS, Sandes AF, et al. Consensus statement for diagnosis and treatment of paroxysmal nocturnal haemoglobinuria. *Hematol Transfus Cell Ther*. 2021;43(3):341-348. doi:10.1016/j.htct.2020.06.006

Effective date: 07/01/2024
Revised date: 12/11/2023