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 Ó10%; 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.