VENTURA COUNTY
HEALTH CARE PLAN

PRIOR AUTHORIZATION POLICY

POLICY: Complement Inhibitors – Fabhalta Prior Authorization Policy
- Fabhalta® (iptacopan capsules – Novartis)

REVIEW DATE: 12/20/2023; selected revision 01/17/2024, 02/28/2024

OVERVIEW

Fabhalta, a Factor B inhibitor, is indicated for the treatment of **paroxysmal nocturnal hemoglobinuria** (PNH) in adults.

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).

Disease Overview

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, genetic disorder of hematopoietic stem cells.[2,3] The mutation in the X-linked gene phosphatidylinositol glycan class A (PIGA) results in a deficiency in the glycosylphosphatidylinositol (GPI) protein, which is responsible for anchoring other protein moieties to the surface of the erythrocytes. Loss of anchoring of these proteins causes cells to hemolyze and leads to complications such as hemolytic anemia, thrombosis, and peripheral blood cytopenias. PNH is a clinical diagnosis that should be confirmed with peripheral blood flow cytometry to detect the absence or severe deficiency of GPI-anchored proteins on at least two lineages.[2,5] Prior to the availability of complement inhibitors, only supportive management, in terms of managing the cytopenias and controlling thrombotic risk were available. Supportive measures include platelet transfusion, immunosuppressive therapy for patients with bone marrow failure, use of erythropoietin for anemias, and aggressive anticoagulation.

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Fabhalta. All approvals are provided for the duration noted below. In cases where the approval is authorized in months, 1 month is equal to 30 days. Because of the specialized skills required for evaluation and diagnosis of patients treated with Fabhalt as well as the monitoring required for adverse events and long-term efficacy, approval requires Fabhalt to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

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

FDA-Approved Indication

1. **Paroxysmal Nocturnal Hemoglobinuria.** Approve for the duration noted if the patient meets ONE of the following (A or B):
   - **A)** Initial therapy. Approve for 6 months if the patient meets the following (i, ii, and iii):
     - **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. The medication is prescribed by or in consultation with a hematologist.

B) Patient is Currently Receiving Fabhalta. Approve for 1 year 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.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Fabhalta is not recommended in the following situations:

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), Soliris (eculizumab intravenous infusion), and Ultomiris (ravulizumab-cwzy intravenous infusion or subcutaneous injection).

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

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.

HISTORY

Type of Revision	Summary of Changes	Review Date
New Policy	--	12/20/2023
Selected Revision	**Conditions Not Recommended for Approval:** Added new criterion regarding concomitant use with another complement inhibitor; examples of complement inhibitors were added as a Note.	01/17/2024
Selected Revision	**Paroxysmal Nocturnal Hemoglobinuria:** Initial approval duration was changed from 4 months to 6 months.	02/28/2024