VENTURA COUNTY
HEALTH CARE PLAN

PRIOR AUTHORIZATION POLICY

POLICY: Complement Inhibitors – Empaveli Prior Authorization Policy
- Empaveli™ (pegcetacoplan subcutaneous infusion – Apellis)

REVIEW DATE: 05/26/2021

OVERVIEW

Empaveli, a complement C3 inhibitor, is indicated for the treatment of **paroxysmal nocturnal hemoglobinuria** (PNH), in adults.

Disease Overview

PNH is a rare disorder involving bone marrow failure that manifests with hemolytic anemia, thrombosis, and peripheral blood cytopenias.[2-4] Due to the absence of two glycosylphosphatidylinositol (GPI)-anchored proteins, CD55 and CD59, uncontrolled complement activation leads to hemolysis and other PNH manifestations. GPI anchor protein deficiency is often due to mutations in phosphatidylinositol glycan class A (PIGA), a gene involved in the first step of GPI anchor biosynthesis. 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.[2-4] Other agents indicated for the management of PNH in adults include Soliris® (eculizumab intravenous infusion) and Ultomiris® (ravulizumab intravenous infusion), both C5 complement inhibitors.[5,6]

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Empaveli. 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 Empaveli as well as the monitoring required for adverse events and long-term efficacy, approval requires Empaveli to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Empaveli 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 4 months if the patient meets the following criteria (i, ii, iii, and iv):
     - **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.** For a patient transitioning to Empaveli from Soliris (eculizumab intravenous infusion) or Ultomiris (ravulizumab intravenous infusion), the prescriber attests that these such medications will be discontinued within 4 weeks after starting Empaveli; AND

iv. Medication is prescribed by, or in consultation with, a hematologist.

B) Patient is Currently Receiving Empaveli: 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 Empaveli 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.** Medication is prescribed by, or in consultation with, a hematologist.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Empaveli is not recommended in the following situations:

1. **Concurrent Use with Soliris (eculizumab intravenous infusion) or Ultomiris (ravulizumab intravenous infusion).** Concurrent use of Soliris and/or Ultomiris, two C5 inhibitors indicated for use in paroxysmal nocturnal hemoglobinuria for adults, with Empaveli is not recommended. However, to reduce the risk of hemolysis from abrupt treatment discontinuation, patients currently receiving Soliris or Ultomiris and switching to Empaveli may receive these agents for no more than 4 weeks after starting Empaveli.

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. Empaveli™ subcutaneous infusion [prescribing information]. Waltham, MA: Apellis; May 2021.
2. Hillmen P, Szer J, Weitz I, et al. Pegcetacoplan versus eculizumab in paroxysmal nocturnal hemoglobinuria. *N Engl J Med*. 2021;384(11):1028-1037.
3. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemogloinuria. *Hematology Am Soc Hematol Edu Program*. 2016;2016(1):208-216.
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.
5. Soliris® intravenous infusion [prescribing information]. Boston, MA: Alexion; November 2020.
6. Ultomiris® intravenous infusion [prescribing information]. Boston, MA: Alexion; October 2020.

HISTORY

Type of Revision	Summary of Changes	Review Date
New Policy	--	05/26/2021