

## **RX.PA.039.MPC Empaveli (pegcetacoplan)**

The purpose of this policy is to define the prior authorization process for Empaveli (pegcetacoplan).

Empaveli (pegcetacoplan) is indicated for the treatment of:

- Adult patients with paroxysmal nocturnal hemoglobinuria (PNH)
- Adult and pediatric patients with C3 glomerulopathy (C3G) to reduce proteinuria
- Adult and pediatric patients with primary immune-complex membranoproliferative glomerulonephritis (IC-MPGN) to reduce proteinuria

### **DEFINITIONS**

**Paroxysmal Nocturnal Hemoglobinuria (PNH)** is a rare blood disorder characterized by destruction of red blood cells, blood clots, and impaired bone marrow function.

**Lactate Dehydrogenase (LDH)** is a catalytic enzyme that is highly concentrated in red blood cells. Increased serum levels of LDH correlate with increased hemolysis of red blood cells.

**PNH clones** are blood cells that are deficient of glycosylphosphatidylinositol-anchored proteins (GPI-AP) due to a genetic mutation of the PIG-A gene in one or more hematopoietic stem cells. This genetic defect causes the blood cells to be attacked by the immune system, resulting in hemolysis, thrombosis, and infection.

### **POLICY**

It is the policy of the Health Plan to maintain a prior authorization process that promotes appropriate utilization of specific drugs with potential for misuse or limited indications. This process involves a review using Food and Drug Administration (FDA) criteria to make a determination of Medical Necessity, and approval by the Pharmacy & Therapeutics Committee of the criteria for prior authorization, as described in RX.002 Pharmacy and Therapeutics Committee and RX.003-Prior Authorization Process.

The drug, Empaveli (pegcetacoplan), is subject to the prior authorization process.

### **PROCEDURE**

#### **1. Initial Authorization Criteria:**

*Must meet all of the criteria listed under the respective diagnosis:*

##### **I. Paroxysmal nocturnal hemoglobinuria (PNH):**

- Must be 18 years of age or older

- Must be prescribed by or in consultation with a hematologist, oncologist, immunologist, or genetic specialist
- Must have a diagnosis of paroxysmal nocturnal hemoglobinuria (PNH) as evidenced by having detectable PNH clones (GPI-AP-deficient hematopoietic clones) via flow cytometry (laboratory documentation must be submitted)
- Must have a lactate dehydrogenase (LDH) level of at least 1.5 times the upper limit of the normal range (laboratory result with reference range must be submitted)
- Must have laboratory documentation of Hemoglobin (Hgb) < 10.5 g/dL (taken within the last 30 days)
- Must provide documentation that a meningococcal vaccine was given at least two (2) weeks prior to the administration of the first dose of Empaveli
- Member must not be using Empaveli concurrently in combination with Soliris or Ultomiris

**II. C3 Glomerulopathy (C3G) or Primary Immune-Complex Membranoproliferative Glomerulonephritis (IC-MPGN):**

- Must be 12 years of age or older
- Must be prescribed by or in consultation with a nephrologist
- Must have a documented diagnosis of C3 glomerulopathy or immune-complex membranoproliferative glomerulonephritis confirmed by kidney biopsy
- Provider and patient must be enrolled in EMPAVELI REMS
- Documented labs within 30 days of request:
  - Proteinuria > 1g/day
  - Urine protein to creatinine ratio (UPCR) > 1g/g
  - Estimated glomerular filtration rate > 30mL/min/1.73m<sup>2</sup>
- Must have a trial of maximally tolerated dose (at least 12 weeks) of an angiotensin converting enzyme inhibitor, angiotensin receptor blocker, or a sodium glucose cotransporter-2 inhibitor unless intolerant or contraindicated to all three classes
- Documentation that the patient will be taking Empaveli in combination with an angiotensin converting enzyme inhibitor or angiotensin receptor blocker unless intolerant or contraindicated
- Must be vaccinated against streptococcus pneumoniae, Neisseria meningitidis, and Haemophilus Influenzae type B at least two (2) weeks prior to the administration of the first dose of Empaveli
- Must not receive Empaveli in combination with another complement inhibitor (Soliris, Ultomiris, Fabhalta, etc.)

**2. Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc.) listed in the FDA approved labeling.**

**3. Empaveli will be considered investigational or experimental for any other use and will not be covered.**

**4. Reauthorization Criteria:**

All prior authorization renewals are reviewed on an annual basis to determine the Medical Necessity for continuation of therapy. Authorization may be extended at 1-year intervals based upon chart documentation from the prescriber that the member's condition has improved based upon the prescriber's assessment while on therapy.

- MPC Renewal:
  - Member has a documented clinical response to therapy as determined by the prescriber.
  - For PNH diagnosis: must be prescribed by or in consultation with a hematologist, oncologist, immunologist, or genetic specialist.
  - For C3G/IC-MPGN diagnosis: must be prescribed by or in consultation with a nephrologist
- Renewal from Previous Insurer:
  - Members who have received prior approval (from insurer other than MPC), or have been receiving medication samples, should be considered under criterion 1 (Initial Authorization Criteria)
  - Provider has a documented clinical response of the member's condition which has stabilized or improved compared to baseline.

**LIMITATIONS**

<b>Length of Authorization (if above criteria met)</b>	
Initial Authorization	Up to 3 months
<b>Reauthorization</b>	
Reauthorization	Up to 1 year
<b>Quantity Level Limit</b>	
Empaveli 1,080 mg/20 mg	8 vials (160 mL) per 28 days

If the established criteria are not met, the request is referred to a Medical Director for review.

## REFERENCES

1. Empaveli (pegcetacoplan) [prescribing information]. Waltham, MA: Apellis Pharmaceuticals, Inc; 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. doi:10.1056/NEJMoa2029073.
3. Cançado RD, Araújo ADS, Sandes AF, et al. Consensus statement for diagnosis and treatment of paroxysmal nocturnal haemoglobinuria [published online ahead of print, 2020 Jul 6]. *Hematol Transfus Cell Ther.* 2020;S2531-1379(20)30079-1. doi:10.1016/j.htct.2020.06.006.
4. Dezern AE, Borowitz MJ. ICCS/ESCCA consensus guidelines to detect GPI-deficient cells in paroxysmal nocturnal hemoglobinuria (PNH) and related disorders part 1 - clinical utility. *Cytometry B Clin Cytom.* 2018;94(1):16-22. doi:10.1002/cyto.b.21608.

## RECORD RETENTION

Records Retention for Evolent Health documents, regardless of medium, are provided within the Evolent Health records retention policy and as indicated in CORP.028.E Records Retention Policy and Procedure.

## REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
<i>Selected Revision</i> <i>Addition of criteria for C3 glomerulopathy (C3G) &amp; Primary Immune-Complex Membranoproliferative Glomerulonephritis (IC-MPGN)</i>	01/2026
<i>Annual Review</i>	02/2025
<i>Annual Review</i> <i>Change in Non-MPC renewal to renewal from previous insurer</i>	02/2024
<i>Annual Review</i>	02/2023
<i>Selected Revision</i> <i>Addition of MPC vs Non-MPC Renewal Criteria</i>	09/2022
<i>Annual Review</i>	02/2022

Empaveli (pegcetacoplan)  
POLICY NUMBER: RX.PA.039.MPC  
REVISION DATE: 01/2026  
PAGE NUMBER: 5 OF 5

<i>Addition of dosing requirements</i>	12/2021
<i>New policy</i>	07/21