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).

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 glycosylphosphatetidylinositol-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 provide documentation that a meningococcal vaccine was given at least two (2) weeks prior to the administration of the first dose of Empaveli
- Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc.) listed in the FDA approved labeling

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.

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 3 months

Reauthorization	Up to 1 year
Quantity Level Limit	
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
Annual Review	*02/2022*
Addition of dosing requirements	*12/2021*
New policy	*07/21*