# Pharmacy Policy Bulletin

**Title:** Pegcetacoplan (Empaveli™)

**Policy #:** Rx.01.252

***Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.***

***This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.***

***Members are advised to use participating pharmacies in order to receive the highest level of benefits.***

**Intent:**

The intent of this policy is to communicate the medical necessity criteria for **Pegcetacoplan (Empaveli™)** as provided under the member's prescription drug benefit.

**Description:**

Paroxysmal nocturnal hemoglobinuria (PNH) is a disorder of the hematopoietic stem cell associated with hemolytic anemia (from uncontrolled complement activation), bone marrow failure, and thrombosis. PNH results from a nonmalignant somatic mutation in the X-linked phosphatidylinositol glycan class A (PIGA) gene in a self-renewing hematopoietic stem cell. The mutation leads to deficiencies in GPI-anchored proteins on hematopoietic cells, including CD55 and CD59. The deficiency of CD55 and CD59 results in activation of the alternative complement pathway. Pegcetacoplan is a complement inhibitor that binds to complement protein C3 and its activation fragment C3b, thereby regulating the cleavage of C3 and the generation of downstream effectors of complement activation.

Empaveli™ is indicated for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH).

**Policy:**

**INITIAL CRITERIA** Pegcetacoplan (Empaveli™) is approved when ALL of the following are met:

1. Diagnosis of paroxysmal nocturnal hemoglobinuria (PNH); and
2. Member is 18 years of age or older; and
3. Member's Hemoglobin (Hb) level is less than 10.5 g/dL despite prior eculizumab (Soliris®) therapy or ravulizumab-cwvz (Ultomiris®) therapy

Initial authorization duration: 12 months

**CONTINUATION CRITERIA** Pegcetacoplan (Empaveli™) is reapproved with documentation of positive clinical response to therapy (e.g., improvement in hemoglobin level, hemoglobin stabilization, decrease in the number of red blood cell transfusions)

Continuation duration: 12 months

**Black Box Warning as shown in the drug Prescribing Information:**

WARNING: SERIOUS INFECTIONS CAUSED BY ENCAPSULATED BACTERIA
See full prescribing information for complete boxed warning.

Meningococcal infections may occur in patients treated with EMPAVELI and may become rapidly life-threatening or fatal if not recognized and treated early. Use of EMPAVELI may predispose individuals to serious infections, especially those caused by encapsulated bacteria, such as Streptococcus pneumoniae, Neisseria meningitidis types A, C, W, Y, and B, and Haemophilus influenzae type B.

EMPAVELI is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS).

**Guidelines:**

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

**BENEFIT APPLICATION**

Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

**References:**

DynaMed [Internet]. Ipswich (MA): EBSCO Information Services. 1995 - . Record No. T115903, Paroxysmal Nocturnal Hemoglobinuria (PNH); [updated 2018 Nov 30, cited 2021 Oct 01]. Available from https://www.dynamed.com/topics/dmp~AN~T115903. Accessed October 01, 2021

Empaveli™ (pegcetacoplan) [package insert]. Waltham (MA): Apellis Pharmaceuticals, Inc. August 2021. Available at: https://dailymed.nlm.nih.gov/dailymed/drugInfo.cfm?setid=c23d89e9-b00b-4520-e053-2995a90a95af. Accessed October 01, 2021.

**Applicable Drugs:**

Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

| Brand Name | Generic Name |
|---|---|
| Empaveli | Pegcetacoplan |

**Cross References:**

Rx.01.33 Off Label Use

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| **Policy Version Number:** | 1.00 |
| **P&T Approval Date:** | September 23, 2021 |
| **Policy Effective Date:** | January 01, 2022 |
| **Next Required Review Date:** | September 23, 2022 |