Empaveli (pegcetacoplan)

COVERAGE CRITERIA

Paroxysmal Nocturnal Hemoglobinuria (PNH)
Pegcetacoplan meets member benefit certificate primary coverage criteria that there be scientific evidence of effectiveness in improving health outcomes for an individual with paroxysmal nocturnal hemoglobinuria (PNH) when ALL the following criteria are met.

INITIAL APPROVAL STANDARD REVIEW for up to 6 months:

1. Individual is 18 years of age or older; **AND**

2. Individual has a diagnosis of PNH confirmed by flow cytometry (Parker, 2016):

   a. Greater than or equal to 10% PNH type III red cells; **OR**
   b. Greater than or equal to 40% Glycosyl phosphatidylinositol–anchored proteins GPI-AP–deficient polymorphonuclear cells (PMNs); **AND**

3. Individual does not have an unresolved serious infection caused by encapsulated bacteria (Empaveli, 2024); **AND**
4. Individual has been immunized against encapsulated bacteria (including Streptococcus pneumoniae, Neisseria meningitidis (serogroups A, C, W, Y, and B), and Haemophilus influenzae type B) at least 2 weeks prior to administration of the first dose of pegcetacoplan unless the clinical record documentation the risks of administration are outweighed by benefit; **AND**

5. Either of the following criteria are met:

   a. Individual is switching from treatment with C5 inhibitor (eculizumab or ravulizumab); **AND**
      i. Individual is currently receiving eculizumab (e.g., Soliris) which will be discontinued after an initial 4 week overlap period with pegcetacoplan; **OR**
      ii. Individual is currently receiving ravulizumab (e.g., Ultomiris) which will be stopped and pegcetacoplan will be initiated no more than 4 weeks after the last dose; **OR**
   b. Individual is C5 Inhibitor naïve (not switching from eculizumab or ravulizumab); **AND**
      i. Individual has documented persistent anemia as defined by the following:
         i. Hemoglobin less than 10.5 g/dL within the last 60 days (Hillmen, 2021); **AND**
         ii. Individual has history of at least 1 transfusion in the last year with documentation (Hillmen, 2021); **AND**
      ii. Individual has lactate dehydrogenase (LDH) greater than or equal to 1.5 times the upper limit of normal, and documentation is provided (Wong, 2023); **AND**

6. Individual will not receive pegcetacoplan in combination with another complement inhibitor or biologic agent (e.g., iptacopan, danicopan, eculizumab, or ravulizumab) used for the treatment of PNH outside of the initial overlap as described in transition of therapy; **AND**

7. Must be dosed in accordance with the FDA label.

CONTINUATION for up to 12 months:
Continuation of therapy for PNH meets primary coverage criteria when:

1. Individual has completed or updated immunization against encapsulated bacteria (including Streptococcus pneumoniae, Neisseria meningitidis (serogroups A, C, W, Y, and B), and Haemophilus influenzae type B); **AND**
2. Individual has experienced a clinical response as documented by one of the following (Hillmen, 2021):

    a. Stabilization of hemoglobin levels; **OR**
    b. Reduction in number of transfusions required; (e.g., 6 months); **AND**
    c. Improvement in hemolysis (i.e., normalization or decrease of LDH levels) (Wong, 2023); **AND**
3. Pegcetacoplan is not being used in combination with another complement inhibitor or biologic agent used for the treatment of PNH.

Dosage and Administration

Dosing per FDA Guidelines

The recommended dose of Pegcetacoplan is 1,080 mg by subcutaneous infusion twice weekly via a commercially available infusion pump with a reservoir of at least 20 mL.

Pegcetacoplan is available as a 1,080 mg/20 mL single-dose vial.

Pegcetacoplan is for subcutaneous infusion using an infusion pump. Pegcetacoplan is intended for use under the guidance of a healthcare professional. After proper training in subcutaneous infusion, an individual may self- administer, or the individual's caregiver may administer pegcetacoplan, if a healthcare provider determines that it is appropriate.

Please refer to separate policy on Site of Care or Site of Service Review (policy #2018030) for pharmacologic/biologic medications.

Does Not Meet Primary Coverage Criteria Or Is Investigational For Contracts Without Primary Coverage Criteria

Pegcetacoplan, for any indication or circumstance not described above, does not meet member benefit certificate primary coverage criteria that there be scientific evidence of effectiveness in improving health outcomes.

For members with contracts without primary coverage criteria, pegcetacoplan, pegcetacoplan, for any indication or circumstance not described above, is considered **investigational**. **Investigational** services are specific contract exclusions in most member benefit certificates of coverage.