CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Empaveli (pegcetacoplan)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Empaveli is the first and only FDA-approved drug for PNH that controls both intravascular and extravascular hemolysis. In contrast to Soliris and Ultomiris, C5 inhibitors which only impact intravascular hemolysis, Empaveli is a C3 inhibitor. The phase 3 PEGASUS study concluded Empaveli was superior to Soliris in terms of improving hemoglobin levels and freedom from transfusion.
PNH is a hematopoietic stem cell disorder in which activation of the complement system destroys red blood cells because of an acquired mutation in the PIGA gene. Common manifestations can include hemolytic anemia and fatigue. Thrombosis and bone marrow suppression may also occur.

Empaveli (pegcetacoplan) will be considered for coverage when the following criteria are met:

Paroxysmal nocturnal hemoglobinuria (PNH)

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a hematologist; AND
3. Member has a documented diagnosis of PNH as confirmed by flow cytometry; AND
4. Member has a lactate dehydrogenase (LDH) level >1.5x upper limit of normal (ULN); AND
5. Member has at least one PNH-related sign/symptom e.g., fatigue, hemoglobin <10 g/dL, thrombosis, pRBC transfusion, shortness of breath; AND
6. Member has been vaccinated against encapsulated bacteria (Streptococcus pneumoniae, Neisseria meningitidis types A, C, W, Y, and B, and Haemophilus influenzae type B).
7. **Dosage allowed/Quantity limit:** 1080 mg subQ twice weekly (via infusion pump or on-body injector). QL 8 vials per 28 days
   Note: May be adjusted to 1080 mg every 3 days if LDH is more than 2x greater than ULN.
   Note: If member is switching from Soliris, it must be discontinued 4 weeks after starting Empaveli.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:
1. Clinical evidence of positive response to therapy such as increased hemoglobin level, decreased need for transfusions, normalized LDH levels, improved fatigue.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Empaveli (pegcetacoplan) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/28/2021	New policy for Empaveli created.
07/27/2023	Added new references. Added requirement that the member must be symptomatic. Moved Soliris note to dosing section. Shortened vaccine requirement statement.
10/13/2023	Added injector to dosing.

References:

1. Empaveli [package insert]. Waltham, MA: Apellis Pharmaceuticals, Inc.; 2023.
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3. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemoglobinuria. *Hematology Am Soc Hematol Educ Program*. 2016;2016(1):208-216. doi:10.1182/asheducation-2016.1.208
4. Patriquin CJ, Kiss T, Caplan S, et al. How we treat paroxysmal nocturnal hemoglobinuria: A consensus statement of the Canadian PNH Network and review of the national registry. *Eur J Haematol*. 2019;102(1):36-52. doi:10.1111/ejh.13176
5. Devos T, Meers S, Boeckx N, et al. Diagnosis and management of PNH: Review and recommendations from a Belgian expert panel. *Eur J Haematol*. 2018;101(6):737-749. doi:10.1111/ejh.13166
6. Bodó I, Amine I, Boban A, et al. Complement Inhibition in Paroxysmal Nocturnal Hemoglobinuria (PNH): A Systematic Review and Expert Opinion from Central Europe on Special Patient Populations. *Adv Ther*. 2023;40(6):2752-2772. doi:10.1007/s12325-023-02510-4
7. Bhak RH, Mody-Patel N, Baver SB, et al. Comparative effectiveness of pegcetacoplan versus ravulizumab in patients with paroxysmal nocturnal hemoglobinuria previously treated with eculizumab: a matching-adjusted indirect comparison. *Curr Med Res Opin*. 2021;37(11):1913-1923. doi:10.1080/03007995.2021.1971182

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