CareSource

PHARMACY POLICY STATEMENT
Nevada Medicaid

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

Empaveli, approved by the FDA in 2021, is a complement protein C3 inhibitor indicated for the treatment of 1) paroxysmal nocturnal hemoglobinuria (PNH) and for 2) C3 glomerulopathy (C3G) or primary immune-complex membranoproliferative glomerulonephritis (IC-MPGN), to reduce proteinuria.
In PNH, Empaveli controls both intravascular and extravascular hemolysis, in contrast to Soliris and Ultomiris, C5 inhibitors which only impact intravascular hemolysis. 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.
The term membranoproliferative glomerulonephritis (MPGN) describes a pattern of injury on a kidney biopsy. It may be seen in the setting of various secondary conditions (e.g., infections, autoimmunity) as well as in primary forms that are distinguished into C3 glomerulopathy (C3G) and immunoglobulin-associated MPGN (Ig-MPGN), based on immunofluorescence. In the Phase 3 VALIANT trial, Empaveli demonstrated a 68% reduction in proteinuria and stabilized kidney function (eGFR) from baseline compared to placebo.

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.

C3 glomerulopathy (C3G) or Primary Immune-complex Membranoproliferative Glomerulonephritis (IC-MPGN)

For **initial** authorization:
1. Member is at least 12 years of age; AND
2. Medication must be prescribed by or in consultation with a nephrologist; AND
3. Member has a documented diagnosis of C3G or IC-MPGN confirmed by renal biopsy; AND
4. Member's UPCR is 1 g/g or greater; AND
5. Member's eGFR is at least 30 mL/min/1.73m^2; AND
6. Member has been on max tolerated dose of an ACEi or ARB for at least 3 months; AND
7. Member has been vaccinated against encapsulated bacteria (Streptococcus pneumoniae, Neisseria meningitidis types A, C, W, Y, and B, and Haemophilus influenzae type B).
8. **Dosage allowed/Quantity limit:**
   Adults: 1080 mg subQ twice weekly
   Pediatric: Twice weekly as follows:

Patient Body Weight	First dose (infusion volume)	Second dose (infusion volume)	Maintenance dose (infusion volume)
50 kg or higher	1,080 mg (20 mL)	1,080 mg (20 mL)	1,080 mg twice weekly (20 mL)
35 kg to less than 50 kg	648 mg (12 mL)	810 mg (15 mL)	810 mg twice weekly (15 mL)
Less than 35 kg	540 mg (10 mL)	540 mg (10 mL)	648 mg twice weekly (12 mL)

   QL 8 vials per 28 days

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

For **reauthorization**:
1. Chart notes must show reduced proteinuria compared to baseline.

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.

07/30/2025	Added new indication for C3G/IC-MPGN.

References:

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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
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10. Noris M, Remuzzi G. C3G and Ig-MPGN-treatment standard. *Nephrol Dial Transplant*. 2024;39(2):202-214. doi:10.1093/ndt/gfad182

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