UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2024 P 2244-4
Program	Prior Authorization/Medical Necessity
Medication	Empaveli® (pegcetacoplan)
P&T Approval Date	7/2021, 7/2022, 8/2023, 2/2024
Effective Date	5/1/2024

1. Background

Empaveli (pegcetacoplan) is a complement inhibitor indicated for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH).[1]

2. Coverage Criteria[a]:

A. Initial Authorization

1. **Empaveli** will be approved based on **all** of the following criteria:

   a. Submission of medical records (e.g., chart notes, laboratory values, etc.) documenting the diagnosis of paroxysmal nocturnal hemoglobinuria (PNH) as confirmed by **both** of the following[2,3,4,5]:

      (1) Flow cytometry analysis confirming presence of PNH clones

      -AND-

      (2) Laboratory results, signs, and/or symptoms attributed to PNH (e.g., abdominal pain, anemia, dyspnea, extreme fatigue, smooth muscle dystonia, unexplained/unusual thrombosis, hemolysis/hemoglobinuria, kidney disease, pulmonary hypertension, etc.)

      -AND-

   b. **Both** of the following:

      (1) Patient will not be prescribed Empaveli in combination with another complement inhibitor used for the treatment of PNH (e.g., Fabhalta, Soliris, Ultomiris)

      -AND-

      (2) **One** of the following:

         (a) Patient is not currently receiving a complement inhibitor medication used for the treatment of PNH (e.g., Fabhalta, Soliris, Ultomiris)

         -OR-

(b) Patient is currently receiving Soliris (eculizumab) which will be discontinued after an initial 4 week overlap period with Empaveli

-OR-

(c) Patient is currently receiving Ultomiris (ravulizumab-cwvz) which will be stopped and Empaveli will be initiated no more than 4 weeks after the last dose

-AND-

c. Prescribed by, or in consultation with one of the following:

(1) Hematologist
(2) Oncologist

Authorization will be issued for 12 months.

B. <u>Reauthorization</u>

1. **Empaveli** will be approved based on **<u>all</u>** of the following criteria:

a. Documentation of positive clinical response to Empaveli therapy (e.g., increased or stabilization of hemoglobin levels, reduction in transfusions, improvement in hemolysis, decrease in LDH, increased reticulocyte count, etc.)

-AND-

b. Patient is not receiving Empaveli in combination with another complement inhibitor used for the treatment of PNH (e.g., Fabhalta, Soliris, Ultomiris)

-AND-

c. Prescribed by, or in consultation with one of the following:

(1) Hematologist
(2) Oncologist

Authorization will be issued for 12 months.

[a] State mandates may apply. Any federal regulatory requirements and the member specific benefit plan coverage may also impact coverage criteria. Other policies and utilization management programs may apply.

3. Additional Clinical Rules:

- Notwithstanding Coverage Criteria, UnitedHealthcare may approve initial and re-authorization based solely on previous claim/medication history, diagnosis codes (ICD-10) and/or claim logic. Use of automated approval and re-approval processes varies by program and/or therapeutic class.
- Supply limits may be in place.

4. References:

1. Empaveli [package insert], Waltham, MA: Apellis Pharmaceuticals, Inc.; September 2023.
2. Parker C, Omine M, Richards S, et al. Diagnosis and management of paroxysmal nocturnal hemoglobinuria. Blood. 2005 Dec 1; 106(12): 3699–3709.
3. Devalet B, Mullier F, Chatelain B, et al. Pathophysiology, diagnosis, and treatment of paroxysmal nocturnal hemoglobinuria: a review. Eur J Haematol. 2015 Sep;95(3):190-8.
4. Sutherland DR, Keeney M, Illingworth A. Practical guidelines for the high-sensitivity detection and monitoring of paroxysmal nocturnal hemoglobinuria clones by flow cytometry. Cytometry B Clin Cytom. 2012 Jul;82(4):195-208.
5. Röth A, Maciejewski J, Nishimura JI, et al. Screening and diagnostic clinical algorithm for paroxysmal nocturnal hemoglobinuria: Expert consensus. Eur J Haematol. 2018 Jul;101(1):3-11.

Program	Prior Authorization/Medical Necessity - Empaveli® (pegcetacoplan)
Change Control	
7/2021	New program
7/2022	Annual review with no changes to coverage criteria. Updated citations in background and coverage criteria.
8/2023	Annual review. Updated references.
2/2024	Added Fabhalta to list of examples of other complement inhibitors used for the treatment of PNH. Revised initial authorization to 12 months. Included criteria for therapeutic duplication. Updated references.