Medical Policy:

Empaveli™ (pegcetacoplan) Subcutaneous

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.340	March 18, 2024	September 14, 2021

The treating physician or primary care provider must submit to EmblemHealth, or ConnectiCare, as applicable (hereinafter jointly referred to as "EmblemHealth"), the clinical evidence that the member meets the criteria for the treatment or surgical procedure. Without this documentation and information, EmblemHealth will not be able to properly review the request preauthorization or post-payment review. The clinical review criteria expressed below reflects how EmblemHealth determines whether certain services or supplies are medically necessary. This clinical policy is not intended to pre-empt the judgment of the reviewing medical director or dictate to health care providers how to practice medicine. Health care providers are expected to exercise their medical judgment in rendering appropriate care. Health care providers are expected to exercise their medical judgment in rendering appropriate care.

EmblemHealth established the clinical review criteria based upon a review of currently available clinical information (including clinical outcome studies in the peer reviewed published medical literature, regulatory status of the technology, evidence-based guidelines of public health and health research agencies, evidence-based guidelines and positions of leading national health professional organizations, views of physicians practicing in relevant clinical areas, and other relevant factors). EmblemHealth expressly reserves the right to revise these conclusions as clinical information changes and welcomes further relevant information. Each benefit program defines which services are covered. The conclusion that a particular service or supply is medically necessary does not constitute a representation or warranty that this service or supply is covered and/or paid for by EmblemHealth, as some programs exclude coverage for services or supplies that EmblemHealth considers medically necessary.

If there is a discrepancy between this guideline and a member's benefits program, the benefits program will govern. Identification of selected brand names of devices, tests and procedures in a medical coverage policy is for reference only and is not an endorsement of any one device, test or procedure over another. In addition, coverage may be mandated by applicable legal requirements of a state, the Federal Government or the Centers for Medicare & Medicaid Services (CMS) for Medicare and Medicaid members. All coding and web site links are accurate at time of publication.

EmblemHealth may also use tools developed by third parties, such as the MCG™ Care Guidelines, to assist us in administering health benefits. The MCG™ Care Guidelines are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice. EmblemHealth Services Company, LLC, has adopted this policy in providing management, administrative and other services to EmblemHealth Plan, Inc., EmblemHealth Insurance Company, EmblemHealth Services Company, LLC, and Health Insurance Plan of Greater New York (HIP) related to health benefit plans offered by these entities. ConnectiCare, an EmblemHealth company, has also adopted this policy. All of the aforementioned entities are affiliated companies under common control of EmblemHealth Inc.

Definitions

Empaveli, a complement C3 inhibitor, is indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH), in adults. PNH is a rare disorder involving bone marrow failure that manifests with hemolytic anemia, thrombosis, and peripheral blood cytopenias. Due to the absence of two glycosylphosphatidylinositol (GPI)-anchored proteins, CD55 and CD59, uncontrolled complement activation leads to hemolysis and other PNH manifestations. GPI anchor protein deficiency is often due to mutations in phosphatidylinositol glycan class A (PIGA), a gene involved in the first step of GPI anchor biosynthesis. PNH is a clinical diagnosis that should be confirmed with peripheral blood flow cytometry to detect the absence or severe deficiency of GPI-anchored proteins on at least two lineages. Other agents indicated for the management of PNH in adults include Soliris® (eculizumab intravenous infusion) and Ultomiris® (ravulizumab intravenous infusion), both C5 complement inhibitors.

Length of Authorization

1. Coverage will be provided for 6 months initially and may be renewed.
2. Continuation approval duration is 1 year.

Dosing Limits [Medical Benefit]

Recommended dosage is 1,080 mg by subcutaneous infusion twice weekly via a commercially available pump.
Max Units (per dose and over time) [HCPCS Unit]:
2,160 mg every 7 days

Guideline

I. Initial Approval Criteria

1. **Paroxysmal nocturnal hemoglobinuria (PNH):**
   Approve if the patient meets all the following criteria:
   A. Patient has a diagnosis of PNH confirmed by peripheral blood flow cytometry results showing the absence or deficiency of glycosylphosphatidylinositol-anchored proteins on at least two cell lineages; **AND**
   B. Patient is 18 years of age or older; **AND**
   C. Prescribed by, or in consultation with, a hematologist; **AND**
   D. Empaveli is being used to treat member's hemolytic anemia due to PNH; **AND**
   E. For a patient transitioning to Empaveli from Soliris (eculizumab intravenous infusion) or Ultomiris (ravulizumab intravenous infusion), the prescriber attests that these such medications will be discontinued within 4 weeks after starting Empaveli.

II. Renewal Criteria:

1. **Paroxysmal nocturnal hemoglobinuria (PNH):**
   A. Patient is responding positively to therapy, as determined by the prescriber; **AND**
   B. Patient has not experienced unacceptable toxicity from the drug.

Limitations/Exclusions

1. Concomitant Use with Soliris (eculizumab intravenous infusion) for > 4 weeks
2. Concomitant Use with Fabhalta (iptacopan capsule) or Ultomiris (ravulizumab intravenous infusion or subcutaneous injection)

Applicable Procedure Codes

Code	Description
C9399	Unclassified drugs or biologicals
J7799	Noc drugs, other than inhalation drugs, administered through dme (infusion pump)

Applicable NDCs

Code	Description
73606-0010-01	Empaveli SubQ solution, 20 ml, 54mg/ml (1080mg/20 ml)

ICD-10 Diagnoses

Code	Description
D59.5	Paroxysmal nocturnal hemoglobinuria

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	3/18/2024	Annual Review: Updated initial length of authorization from 4 months to 6 months. Updated dosing limits. Initial Criteria: PNH: added: "confirmed by peripheral blood flow cytometry results showing the absence or deficiency of glycosylphosphatidylinositol-anchored proteins on at least two cell lineages" to diagnosis. Added Limitations and Exclusions.
EmblemHealth & ConnectiCare	07/06/2023	Annual Review: No criteria updates Removed code J3490, added J7799
EmblemHealth & ConnectiCare	04/21/2022	Transferred policy to new template
EmblemHealth & ConnectiCare	09/14/2021	New Policy

References

1. EMPAVELI™ [package insert].Waltham, MA. Apellis Pharmaceuticals, Inc. Updated July 2021. Accessed July 28, 2021.
2. EMPAVELI ™ Injection. IBM Micromedex® [database online]. Greenwood Village, CO. Truven Health Analytics. Available at: https://www.micromedexsolutions.com. Updated June 21, 2021. Accessed July 28, 2021.
3. Hillmen P, Szer J, Weitz I, et al. Pegcetacoplan versus eculizumab in paroxysmal nocturnal hemoglobinuria. N Engl J Med. 2021;384(11):1028-1037.
4. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemogloinuria. Hematology Am Soc Hematol Edu Program. 2016;2016(1):208-216.
5. Roth A, Maciejewski J, Nishinura JI, et al. Screening and diagnostic clinical algorithm for paroxysmal nocturnal hemoglobinuria: Expert consensus. Eur J Haematol. 2018;101(1):3-11.
6. Soliris® intravenous infusion [prescribing information]. Boston, MA: Alexion; November 2020.
7. Ultomiris® intravenous infusion [prescribing information]. Boston, MA: Alexion; October 2020.