POLICY Document for EMPAVELI (pegcetacoplan)

The overall objective of this policy is to support the appropriate and cost-effective use of the medication, specific to use of preferred medication options, and overall, clinically appropriate use. This document provides specific information to both sections of the overall policy.

Section 1: Preferred Product
- Policy information specific to preferred medications

Section 2: Clinical Criteria
- Policy information specific to the clinical appropriateness for the medication

Section 1: Preferred Product

CAREFIRST: EXCEPTIONS CRITERIA
COMPLEMENT INHIBITORS

PREFERRED PRODUCTS: ULTOMIRIS, VYVGART, VYVGART HYTRULO

Client Requested: The intent of the criteria is to ensure that patients follow selection elements as established by CareFirst.

POLICY
This policy informs prescribers of preferred products and provides an exception process for targeted products through prior authorization.

I. PLAN DESIGN SUMMARY

This program applies to the Complement inhibitor products specified in this policy. Coverage for targeted products is provided based on clinical circumstances that would exclude the use of the preferred product and may be based on previous use of a product. The coverage review process will ascertain situations where a clinical exception can be made. This program applies to all members requesting treatment with a targeted product.

Each referral is reviewed based on all utilization management (UM) programs implemented for the client.

Table. Complement Inhibitor Products

	Product(s)
Preferred*	• **Ultomiris** (ravulizumab-cwvz) • **Vyvgart** (efgartigimod alfa) • **Vyvgart** Hytrulo (efgartigimod alfa and hyaluronidase)
Targeted	• **Empaveli** (pegcetacoplan) • **Enspryng** (atralizumab-mwge) • **Piasky** (crovalimab) • **Rystiggo** (rozanolixizumab) • **Soliris** (eculizumab) • **Uplizna** (inebilizumab-cdon)

*: Medications considered formulary or preferred on your plan may still require a clinical prior authorization review.

II. EXCEPTION CRITERIA

This program applies to members requesting treatment for an indication that is FDA-approved for the preferred product.

For Myasthenia Gravis, coverage for the targeted product is provided when member has a documented inadequate response, contraindication, or intolerable adverse event to all the preferred products.

For all other indications, coverage for the targeted product is provided when member has a documented inadequate response, contraindication, or intolerable adverse event to Ultomiris.

Section 2: Clinical Criteria

SPECIALTY GUIDELINE MANAGEMENT

EMPAVELI (pegcetacoplan)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Empaveli is indicated for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH).

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review for new requests for treatment of:

A. For initial requests: flow cytometry used to show results of glycosylphosphatidylinositol-anchored proteins (GPI-APs) deficiency.
B. For continuation requests: Chart notes or medical record documentation supporting positive clinical response.

III. CRITERIA FOR INITIAL APPROVAL

Paroxysmal nocturnal hemoglobinuria
Authorization of 6 months may be granted for treatment of paroxysmal nocturnal hemoglobinuria (PNH) when all of the following criteria are met:

A. The diagnosis of PNH was confirmed by detecting a deficiency of glycosylphosphatidylinositol-anchored proteins (GPI-APs) as demonstrated by either of the following:
    1. At least 5% PNH cells
    2. At least 51% of GPI-AP deficient poly-morphonuclear cells
B. Flow cytometry is used to demonstrate GPI-APs deficiency

IV. CONTINUATION OF THERAPY

Paroxysmal nocturnal hemoglobinuria
Authorization of 12 months may be granted for continued treatment in members requesting reauthorization when there is no evidence of unacceptable toxicity or disease progression while on the current regimen and demonstrate a positive response to therapy (e.g., improvement in hemoglobin levels, normalization of lactate dehydrogenase [LDH] levels).

V. DOSAGE AND ADMINISTRATION

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

REFERENCES:

SECTION 1

1. Empaveli [package insert]. Waltham, MA: Apellis Pharmaceuticals, Inc; February 2024.
2. Enspryng [package insert]. San Francisco, CA: Genentech, Inc.; March 2022.
3. Piasky [package insert]. South San Francisco, CA: Genetech Inc; June 2024.
4. Rystiggo [package insert]. Smyrna, GA: UCB, Inc; June 2023.
5. Soliris [package insert]. Boston, MA: Alexion Pharmaceuticals Inc; September 2024.
6. Ultomiris [package insert]. Boston, MA: Alexion Pharmaceuticals Inc; September 2024.
7. Uplinza [package insert]. Deerfield, IL: Horizon Therapeutics; July 2021.
8. Vyvgart [package insert]. Boston, MA: argenx US, Inc; August 2024
9. Vyvgart Hytrulo [package insert]. Boston, MA: argenx US, Inc; August 2024

SECTION 2

1. Empaveli [package insert]. Waltham, MA: Apellis Pharmaceuticals, Inc.; February 2023
2. Parker CJ. Management of paroxysmal nocturnal hemoglobinuria in the era of complement inhibitory therapy. *Hematology.* 2011; 21-29.
3. Borowitz MJ, Craig F, DiGiuseppe JA, et al. Guidelines for the Diagnosis and Monitoring of Paroxysmal Nocturnal Hemoglobinuria and Related Disorders by Flow Cytometry. *Cytometry B Clin Cytom*. 2010: 78: 211-230.
4. Preis M, Lowrey CH. Laboratory tests for paroxysmal nocturnal hemoglobinuria (PNH). Am J Hematol. 2014;89(3):339-341.
5. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemoglobinuria. Hematology Am Soc Hematol Educ Program. 2016;2016(1):208-216.
6. Dezern AE, Borowitz MJ. ICCS/ESCCA consensus guidelines to detect GPI-deficient cells in paroxysmal nocturnal hemoglobinuria (PNH) and related disorders part 1 - clinical utility. Cytometry B Clin Cytom. 2018 Jan;94(1):16-22.