CarePartners of Connecticut MEDICARE ADVANTAGE

Medical Necessity Guidelines

Medical Benefit Drugs

Empaveli™ (pegcetacoplan)

Effective: October 1, 2023

Guideline Type	☒ Prior Authorization ☐ Non-Formulary ☐ Step-Therapy ☐ Administrative

Applies to:

☒ CarePartners of Connecticut Medicare Advantage HMO plans, Fax 617-673-0956

☒ CarePartners of Connecticut Medicare Advantage PPO plans, Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Approval of Empaveli was based on the Phase 3 PEGASUS trial in 80 adults with PNH comparing Empaveli to Soliris. Data demonstrated that Empaveli was superior to Soliris in improving hemoglobin levels and freedom from transfusion in patients with PNH who had hemoglobin levels <10.5 g/dL despite treatment with Soliris. Noninferiority was demonstrated in the endpoints of transfusion avoidance and change from baseline in absolute reticulocyte count. Noninferiority was not shown for the change in baseline in lactate dehydrogenase (LDH) levels. The FACIT-F scores increased in Empaveli-treated patients compared to Soliris-treated patients; however, noninferiority was not assessed.

The Phase 3 PRINCE trial tested Empaveli in treatment-naive PNH patients. Data demonstrated that Empaveli was statistically superior for the co-primary endpoints of hemoglobin stabilization and LDH reduction compared with standard of care. After 26 weeks of treatment, 86% of Empaveli-treated patients avoided a 1 g/dL decrease in hemoglobin levels without transfusions compared with 0% of patients receiving standard of care. Mean LDH in Empaveli-treated patients decreased by 90% from a baseline of 2151 U/L compared with a 14% reduction in patients receiving standard of care.

Food and Drug Administration-Approved Indications

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

Clinical Guideline Coverage Criteria

The Plan may authorize coverage of Empaveli for Members when the following criteria is met:

1. Documented diagnosis of paroxysmal nocturnal hemoglobinuria

Limitations

None

Codes

The following code(s) require prior authorization:

Table 1: HCPCS Codes

HCPCS Codes	Description
C9151	Injection, pegcetacoplan, 1 mg

References

1. Empaveli (pegcetacoplan) [package insert]. Waltham, MA: Apellis Pharmaceuticals, Inc.; Feb 2023.
2. Hillmen P, et al. Pegcetacoplan versus eculizumab in paroxysmal nocturnal hemoglobinuria. N Engl J Med. 2021;384:1028-037.
3. Peffault de Latour R, et al. Forty-Eight Week Efficacy and Safety of Pegcetacoplan in Adult Patients with Paroxysmal Nocturnal Hemoglobinuria and Suboptimal Response to Prior Eculizumab Treatment. Abstract S174. EHA 2021.
4. Wong R, et al. Pegcetacoplan controls hemolysis in complement inhibitor–naive patients with paroxysmal nocturnal hemoglobinuria. Blood Adv. 2023 Jun 13;7(11):2468-78.

Approval And Revision History

September 13, 2022: Reviewed by Pharmacy and Therapeutics Committee (P&T).

September 21, 2022: Reviewed by the Medical Policy Approval Committee (MPAC).

Subsequent endorsement date(s) and changes made:

- Originally approved September 13, 2022, by P&T and September 21, 2022 by MPAC committees effective January 1, 2023
- Administrative update: June 2023 added Medical Benefit Drugs to title and CPCT logo was updated
- Coding update per HCPCS level II quarterly release. Effective date July 1, 2023, the following HCPCS codes have been added: C9151
- September 12, 2023: Removed the Limitation “The Plan considers Empaveli as experimental/investigational and not medically necessary for all other indications.” Removed the Step Therapy requirements. Removed age requirements (effective 10/1/23).
- November 2023: Administrative Update in support of calendar year 2024 Medicare Advantage and PDP Final Rule.

Background, Product and Disclaimer Information

Point32Health prior authorization criteria to be applied to Medicare Advantage plan members is based on guidance from Medicare laws, National Coverage Determinations (NCDs) or Local Coverage Determinations (LCDs). When no guidance is provided, Point32Health uses clinical practice guidance published by relevant medical societies, relevant medical literature, Food and Drug Administration (FDA)-approved package labeling, and drug compendia to develop prior authorization criteria to apply to Medicare Advantage plan members. Medications that require prior authorization generally meet one or more of the following criteria: Drug product has the potential to be used for cosmetic purposes; drug product is not considered as first-line treatment by medically accepted practice guidelines, evidence to support the safety and efficacy of a drug product is poor, or drug product has the potential to be used for indications outside of the indications approved by the FDA. Prior authorization and use of the coverage criteria within this Medical Necessity Guideline will ensure drug therapy is medically necessary, clinically appropriate, and aligns with evidence-based guidelines. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests revisions.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guidelines not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.