PHARMACY POLICY STATEMENT
Marketplace

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

Empaveli is the first and only FDA-approved drug for PNH that controls both intravascular and extravascular hemolysis. In contrast to Soliris and Ultomiris, C5 inhibitors which only impact intravascular hemolysis, Empaveli is a C3 inhibitor. 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.

Empaveli (pegcetacoplan) will be considered for coverage when the following criteria are met:

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.

References:

1. Empaveli [package insert]. Waltham, MA: Apellis Pharmaceuticals, Inc.; 2023.
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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
5. Devos T, Meers S, Boeckx N, et al. Diagnosis and management of PNH: Review and recommendations from a Belgian expert panel. *Eur J Haematol*. 2018;101(6):737-749. doi:10.1111/ejh.13166
6. Bodó I, Amine I, Boban A, et al. Complement Inhibition in Paroxysmal Nocturnal Hemoglobinuria (PNH): A Systematic Review and Expert Opinion from Central Europe on Special Patient Populations. *Adv Ther*. 2023;40(6):2752-2772. doi:10.1007/s12325-023-02510-4
7. Bhak RH, Mody-Patel N, Baver SB, et al. Comparative effectiveness of pegcetacoplan versus ravulizumab in patients with paroxysmal nocturnal hemoglobinuria previously treated with eculizumab: a matching-adjusted indirect comparison. *Curr Med Res Opin*. 2021;37(11):1913-1923. doi:10.1080/03007995.2021.1971182

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