BlueCross BlueShield Federal Employee Program

EMPAVELI
(pegcetacoplan)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Empaveli (pegcetacoplan) is a complement inhibitor indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Empaveli binds to complement protein C3 and its activation fragment C3b, thereby regulating the cleavage of C3 and the generation of downstream effectors of complement activation. In paroxysmal nocturnal hemoglobinuria (PNH), extravascular hemolysis (EVH) is facilitated by C3b opsonization while intravascular hemolysis (IVH) is mediated by downstream membrane attack complex. Empaveli acts proximally in the complement cascade controlling both C3b-mediated EVH and terminal complement-mediated IVH (1).

Regulatory Status

FDA-approved indication: Empaveli is a complement inhibitor indicated for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH) (1).

Empaveli has a boxed warning regarding serious infections caused by encapsulated bacteria. Infections caused by encapsulated bacteria, such as *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type B may occur in patients treated with Empaveli and may become rapidly life-threatening or fatal if not recognized and treated early. Patients should be vaccinated against encapsulated bacteria at least 2 weeks prior to initiation of Empaveli therapy according to current Advisory Committee on Immunization Practices (ACIP) guidelines. Because of the risk of serious infections caused by encapsulated bacteria, Empaveli is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the Empaveli REMS. Under the Empaveli REMS, prescribers must enroll in the program (1).

Empaveli also has warnings regarding infusion-related reactions, monitoring PNH manifestations after discontinuation of Empaveli, and interference of laboratory tests (1).

Empaveli may cause embryo-fetal harm when administered to a pregnant woman. Pregnancy testing is recommended for females of reproductive potential prior to treatment with Empaveli. Female patients of reproductive potential should be advised to use effective contraception during treatment with Empaveli and for 40 days after the last dose (1).

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The safety and effectiveness of Empaveli in pediatric patients less than 18 years of age have not been established (1).

Summary

Empaveli (pegcetacoplan) is a complement inhibitor indicated for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Empaveli has a boxed warning citing the risk of serious infections caused by encapsulated bacteria and it is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Empaveli also has warnings regarding infusion-related reactions, monitoring PNH manifestations after discontinuation of Empaveli, and interference of laboratory tests. The safety and effectiveness of Empaveli in pediatric patients less than 18 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Empaveli while maintaining optimal therapeutic outcomes.

References

1. Empaveli [package insert]. Waltham, MA: Apellis Pharmaceuticals, Inc.; February 2024.