

Sample Letter of Medical Necessity Template for EMPAVELI® (pegcetacoplan)

[Note: When preparing the actual letter, use professional/physician letterhead.]

This sample letter is for informational purposes only. Use of this information does not constitute medical or legal advice and does not guarantee coverage. It is not intended to be a substitute for, or an influence on, the independent clinical decision of the prescriber.

Please see Important Safety Information on pages 2-3, and full [Prescribing Information](#), including Boxed WARNING regarding serious infections caused by encapsulated bacteria, and [Medication Guide](#).

[Date]

[Payer Medical/Pharmacy Director/Contact Name]

[Payer Organization Name]

[Payer Street Address]

[Payer City, State, ZIP Code]

RE: [Patient Name]

Date of Birth: [Patient Date of Birth]

Policy ID/Group Number: [Policy ID/Group Number]

Policy Holder: [Policy Holder's Name]

Dear [Payer Medical/Pharmacy Director/Contact Name]:

I am [Physician Name, Credentials, Specialty, Hospital/Practice]. I am writing on behalf of my patient, [Patient Name], to document the medical necessity for treatment with EMPAVELI® (pegcetacoplan) for the diagnosis of paroxysmal nocturnal hemoglobinuria (PNH). This letter provides information about [Patient Name]'s medical history, diagnosis, prognosis, and treatment plan with EMPAVELI.

1. Summary of Patient's Medical History

[You may be required to include the following:]

- *Patient's diagnosis of PNH and date of diagnosis*
- *Relevant medical history*
- *Patient's current condition*
- *Prior treatments for PNH and response to those treatments*

2. Patient-Specific Rationale for Treatment

[Insert summary statement for treatment rationale as well as summary of your professional opinion of the patient's anticipated prognosis or disease progression without treatment with EMPAVELI.]

Prescriber may choose to include the specific criteria for coverage that the patient meets based on the patient's health plan, along with other relevant details.]

Please call my office at [Telephone Number] if I can provide you with any additional information. I look forward to receiving your timely response and approval of this authorization.

Sincerely,

[Physician Name and Participating Provider Number]

INDICATION

EMPAVELI® (pegcetacoplan) is indicated for the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH).

IMPORTANT SAFETY INFORMATION

WARNING: SERIOUS INFECTIONS CAUSED BY ENCAPSULATED BACTERIA

Meningococcal infections may occur in patients treated with EMPAVELI and may become rapidly life-threatening or fatal if not recognized and treated early. Use of EMPAVELI may predispose individuals to serious infections, especially those caused by encapsulated bacteria, such as *Streptococcus pneumoniae*, *Neisseria meningitidis* types A, C, W, Y, and B, and *Haemophilus influenzae* type B.

- Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for vaccinations against encapsulated bacteria.
- Vaccinate patients at least 2 weeks prior to administering the first dose of EMPAVELI unless the risks of delaying therapy with EMPAVELI outweigh the risk of developing a serious infection.
- Vaccination reduces, but does not eliminate, the risk of serious infections. Monitor patients for early signs of serious infections and evaluate immediately if infection is suspected.

EMPAVELI is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the EMPAVELI REMS, prescribers must enroll in the program.

CONTRAINDICATIONS

- Hypersensitivity to pegcetacoplan or to any of the excipients
- Not currently vaccinated against certain encapsulated bacteria, unless the risks of delaying EMPAVELI treatment outweigh the risks of developing a bacterial infection with an encapsulated organism
- Unresolved serious infection caused by encapsulated bacteria including *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae*

WARNINGS AND PRECAUTIONS

Serious Infections Caused by Encapsulated Bacteria

The use of EMPAVELI may predispose individuals to serious, life-threatening, or fatal infections caused by encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis* types A, C, W, Y, and B, and *Haemophilus influenzae* type B (Hib). To reduce the risk of infection, all patients must be vaccinated against these bacteria according to the most current ACIP recommendations for patients with altered immunocompetence associated with complement deficiencies. Revaccinate patients in accordance with ACIP recommendations considering the duration of therapy with EMPAVELI.

For patients without known history of vaccination, administer required vaccines at least 2 weeks prior to receiving the first dose of EMPAVELI. If immediate therapy with EMPAVELI is indicated, administer required vaccine as soon as possible and provide patients with 2 weeks of antibacterial drug prophylaxis.

Closely monitor patients for early signs and symptoms of serious infection and evaluate patients immediately if an infection is suspected. Promptly treat known infections. Serious infection may become rapidly life-threatening or fatal if not recognized and treated early. Consider discontinuation of EMPAVELI in patients who are undergoing treatment for serious infections.

IMPORTANT SAFETY INFORMATION (cont'd)

WARNINGS AND PRECAUTIONS (cont'd)

EMPAVELI REMS

Because of the risk of serious infections, EMPAVELI is available only through a restricted program under a REMS. Under the EMPAVELI REMS, prescribers must enroll in the program and must counsel patients about the risk of serious infection, provide the patients with the REMS educational materials, and ensure patients are vaccinated against encapsulated bacteria. Enrollment and additional information are available by telephone: 1-888-343-7073 or at www.empavelirems.com.

Infusion-Related Reactions

Systemic hypersensitivity reactions (e.g., facial swelling, rash, urticaria) have occurred in patients treated with EMPAVELI. One patient (less than 1% in clinical studies) experienced a serious allergic reaction which resolved after treatment with antihistamines. If a severe hypersensitivity reaction (including anaphylaxis) occurs, discontinue EMPAVELI infusion immediately, institute appropriate treatment, per standard of care, and monitor until signs and symptoms are resolved.

Monitoring PNH Manifestations after Discontinuation of EMPAVELI

After discontinuing treatment with EMPAVELI, closely monitor for signs and symptoms of hemolysis, identified by elevated LDH levels along with sudden decrease in PNH clone size or hemoglobin, or reappearance of symptoms such as fatigue, hemoglobinuria, abdominal pain, dyspnea, major adverse vascular events (including thrombosis), dysphagia, or erectile dysfunction. Monitor any patient who discontinues EMPAVELI for at least 8 weeks to detect hemolysis and other reactions. If hemolysis, including elevated LDH, occurs after discontinuation of EMPAVELI, consider restarting treatment with EMPAVELI.

Interference with Laboratory Tests

There may be interference between silica reagents in coagulation panels and EMPAVELI that results in artificially prolonged activated partial thromboplastin time (aPTT); therefore, avoid the use of silica reagents in coagulation panels.

ADVERSE REACTIONS

Most common adverse reactions in patients with PNH (incidence $\geq 10\%$) were injection-site reactions, infections, diarrhea, abdominal pain, respiratory tract infection, pain in extremity, hypokalemia, fatigue, viral infection, cough, arthralgia, dizziness, headache, and rash.

USE IN SPECIFIC POPULATIONS

Females of Reproductive Potential

EMPAVELI may cause embryo-fetal harm when administered to pregnant women. Pregnancy testing is recommended for females of reproductive potential prior to treatment with EMPAVELI. Advise female patients of reproductive potential to use effective contraception during treatment with EMPAVELI and for 40 days after the last dose.

Please see full [Prescribing Information](#), including **Boxed WARNING regarding serious infections caused by encapsulated bacteria, and [Medication Guide](#).**

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