

Sample Letter of Medical Necessity Template for EMPAVELI® (pegcetacoplan) for Paroxysmal Nocturnal Hemoglobinuria (PNH)

[Note: When preparing the actual letter, use professional/physician letterhead and ensure it is signed by the prescriber.]

INTRODUCTION

A letter of medical necessity explains why EMPAVELI is medically appropriate for a specific patient. It may be submitted as part of a prior authorization request or in response to a request for additional documentation. The letter should include patient-specific documentation (eg, medical records, clinical treatment history) and other supporting information (eg, the Prescribing Information, relevant peer-reviewed literature).

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. Health plan requirements may vary. Please confirm the specific information required by your patient's health plan to ensure you are providing accurate and complete information.

Please see Important Safety Information on pages 3-5, 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 First Name] [Patient Last Name]

Patient Date of Birth: [Patient Date of Birth]

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

Policy Holder: [Policy Holder's Name]

Diagnosis: [ICD-10-CM Code] [Diagnosis]

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

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

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 (including signs of disease progression that led to you prescribing EMPAVELI)
- Current and prior treatments for PNH and response to those treatments]

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.]

Based on the above, it is my medical opinion that EMPAVELI is appropriate and necessary to treat [Patient Name]'s PNH. 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 request.

Sincerely,

[Physician Name], [MD] or [DO]

[Participating Provider Number]

Enclosures

[The following is a suggested list of enclosures. Please confirm the specific information required by your patient's health plan to ensure you are providing accurate and complete information.

- Supporting clinical documentation
- Copy of patient's insurance card
- Relevant peer-reviewed articles
- A copy of the health plan medical policy, if available
- EMPAVELI Prescribing Information]

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

EMPAVELI, a complement inhibitor, increases the risk of serious infections, especially those caused by encapsulated bacteria, such as *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type B. Life-threatening and fatal infections with encapsulated bacteria have occurred in patients treated with complement inhibitors. These infections may become rapidly life-threatening or fatal if not recognized and treated early.

- Complete or update vaccination for encapsulated bacteria at least 2 weeks prior to the first dose of EMPAVELI, unless the risks of delaying therapy with EMPAVELI outweigh the risks of developing a serious infection. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for vaccinations against encapsulated bacteria in patients receiving a complement inhibitor.
- Patients receiving EMPAVELI are at increased risk for invasive disease caused by encapsulated bacteria, even if they develop antibodies following vaccination. Monitor patients for early signs and symptoms of serious infections and evaluate immediately if infection is suspected.

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.

CONTRAINDICATIONS

- Hypersensitivity to pegcetacoplan or to any of the excipients
- For initiation in patients with unresolved serious infection caused by encapsulated bacteria including *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type B

WARNINGS AND PRECAUTIONS

Serious Infections Caused by Encapsulated Bacteria

EMPAVELI, a complement inhibitor, increases a patient's susceptibility to serious, life-threatening, or fatal infections caused by encapsulated bacteria including *Streptococcus pneumoniae*, *Neisseria meningitidis* (caused by any serogroup, including non-groupable strains), and *Haemophilus influenzae* type B. Life-threatening and fatal infections with encapsulated bacteria have occurred in both vaccinated and unvaccinated patients treated with complement inhibitors. The initiation of EMPAVELI treatment is contraindicated in patients with unresolved serious infection caused by encapsulated bacteria.

Complete or update vaccination against encapsulated bacteria at least 2 weeks prior to administration of the first dose of EMPAVELI, according to the most current ACIP recommendations for patients receiving a complement inhibitor. Revaccinate patients in accordance with ACIP recommendations considering the duration of therapy with EMPAVELI. Note that, ACIP recommends an administration schedule in patients receiving complement inhibitors that differs from the administration schedule in the vaccine prescribing information. If urgent EMPAVELI therapy is indicated in a patient who is not up to date with vaccines against encapsulated bacteria according to ACIP recommendations, provide the patient with antibacterial drug prophylaxis and administer these vaccines as soon as possible. The benefits and risks of treatment with EMPAVELI, as well as the benefits and risks of antibacterial drug prophylaxis in unvaccinated or vaccinated patients, must be considered against the known risks for serious infections caused by

encapsulated bacteria.

Vaccination does not eliminate the risk of serious encapsulated bacterial infections, despite development of antibodies following vaccination. Closely monitor patients for early signs and symptoms of serious infection and evaluate patients immediately if an infection is suspected. Inform patients of these signs and symptoms and instruct patients to seek immediate medical care if these signs and symptoms occur. Promptly treat known infections. Serious infection may become rapidly life-threatening or fatal if not recognized and treated early. Consider interruption of EMPAVELI in patients who are undergoing treatment for serious infections.

EMPAVELI is available only through a restricted program under a REMS.

EMPAVELI REMS

EMPAVELI is available only through a restricted program under a REMS called EMPAVELI REMS, because of the risk of serious infections caused by encapsulated bacteria. Notable requirements of the EMPAVELI REMS include the following:

Under the EMPAVELI REMS, prescribers must enroll in the program. Prescribers must counsel patients about the risks, signs, and symptoms of serious infections caused by encapsulated bacteria, provide patients with the REMS educational materials, ensure patients are vaccinated against encapsulated bacteria at least 2 weeks prior to the first dose of EMPAVELI, prescribe antibacterial drug prophylaxis if patients' vaccine status is not up to date and treatment must be started urgently, and provide instructions to always carry the Patient Safety Card both during treatment, as well as for 2 months following last dose of EMPAVELI. Pharmacies that dispense EMPAVELI must be certified in the EMPAVELI REMS and must verify prescribers are certified.

Further information is available at www.empavelirems.com or 1-888-343-7073.

Infusion-Related Reactions

Systemic hypersensitivity reactions (e.g., facial swelling, rash, urticaria) have occurred in patients treated with EMPAVELI, which may resolve after treatment with antihistamines. Cases of anaphylaxis leading to treatment discontinuation have been reported. 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.

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