Paroxysmal Nocturnal Hemoglobinuria (PNH) Alternative Pathway Complement Inhibitors: Pegcetacoplan (EMPAVELI), Iptacopan (FABHALTA), Danicopan (VOYDEYA) Criteria for Use December 2024

VA Pharmacy Benefits Management Services and National Formulary Committee

The following recommendations are based on medical evidence, clinician input, and expert opinion. The content of the document is dynamic and will be revised as new information becomes available. The purpose of this document is to assist practitioners in clinical decision-making, to standardize and improve the quality of patient care, and to promote cost-effective drug prescribing. THE CLINICIAN SHOULD USE THIS GUIDANCE AND INTERPRET IT IN THE CLINICAL CONTEXT OF THE INDIVIDUAL PATIENT. INDIVIDUAL CASES THAT ARE EXCEPTIONS TO THE EXCLUSION AND INCLUSION CRITERIA SHOULD BE ADJUDICATED AT THE LOCAL FACILITY ACCORDING TO THE POLICY AND PROCEDURES OF ITS P&T COMMITTEE AND PHARMACY SERVICES.

The Product Information should be consulted for detailed prescribing information.

See the VA National Formulary Committee Monograph on this drug at the *PBM INTRAnet* site for further information.

Exclusion Criteria

If the answer to ANY item below is met, then the patient should NOT receive pegcetacoplan/iptacopan/danicopan.

☐ *Neisseria meningitidis, Neisseria gonorrhoeae, Haemophilus influenzae*, or *Streptococcus pneumoniae* active infection

Inclusion Criteria

All of the following criteria must be met.

☐ Must be prescribed by a REMS registered VA or VA Community Care hematologist, oncologist, immunologist or genetic specialist

☐ Laboratory-confirmed diagnosis of Paroxysmal Nocturnal Hemoglobinuria (PNH), as evidenced by detectable GPI-deficient hematopoietic clones (Type III PNH red blood cells (RBC)) via Flow Cytometry.

☐ Evidence of clinically significant hemolysis (e.g., Hemoglobin <10g/dL) despite 6 months of stable therapy with anti-C5 inhibitor (e.g., ravulizumab or eculizumab)

☐ Complete or update vaccination for encapsulated bacteria, including Streptococcus pneumoniae and Neisseria meningitidis at least 2 weeks prior to the first dose of therapy according to current Advisory Committee on Immunization Practices (ACIP)

Other Justification

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