Pharmacy Policy Bulletin: J-1383 Voydeya (danicopan) – Commercial and Healthcare Reform	
Number: J-1383	**Category:** Prior Authorization
Line(s) of Business: ☒ Commercial ☒ Healthcare Reform ☐ Medicare	**Benefit(s):** **Commercial:** **Prior Authorization (1.):** **1.** Miscellaneous Specialty Drugs Oral = Yes w/ Prior Authorization **Healthcare Reform:** Not Applicable
Region(s): ☒ All ☐ Delaware ☐ New York ☐ Pennsylvania ☐ West Virginia	**Additional Restriction(s):** None
Version: J-1383-002	**Original Date:** 06/05/2024
Effective Date: 07/18/2025	**Review Date:** 6/25/2025

Drugs Product(s):	• Voydeya (danicopan)
FDA-Approved Indication(s):	• Add-on therapy to ravulizumab or eculizumab for the treatment of extravascular hemolysis (EVH) in adults with paroxysmal nocturnal hemoglobinuria (PNH).

Background:	• PNH is a rare, acquired hematopoietic stem cell disorder caused by a mutation of the phosphatidylinositol glycan class A (*PIGA*) gene in bone marrow stem cells. *PIGA*-mutant (PNH) cells lack all glycophosphatidyl inositol (GPI) anchored proteins, including CD55 and CD59, two essential complement regulatory proteins. CD59 deficiency on the surfaces of red blood cells (RBCs) causes intravascular hemolysis (IVH) by uncontrolled C5 activation in the terminal complement pathway, and accounts for most PNH manifestations. IVH can lead to thrombosis, which in turn causes much of the morbidity and mortality associated with PNH. CD55 deficiency leads to extravascular hemolysis (EVH) in organs like the spleen by uncontrolled C3 activation in the proximal complement pathway. Although EVH is not life-threatening, it can result in anemia requiring blood transfusions. • Patients with PNH may present with one or more of the following signs and symptoms: fatigue, dyspnea, hemoglobinuria, abdominal pain, bone marrow suppression, erectile dysfunction, chest pain, thrombosis, and renal insufficiency. Thrombosis is the leading cause of death in patients with PNH. • Since the only cure for PNH is allogeneic hematopoietic stem cell transplant, current treatment strategy focuses on managing the various disease symptoms with complement-inhibitory drugs. • Voydeya works by selectively inhibiting Factor D (FD), a complement system protein that plays a key role in the amplification of the complement system response. • Prescribing Considerations:

	○ Dose starts at 150 mg orally three times a day, with or without food. Depending on the clinical response, can increase to 200 mg orally three times a day. ○ Voydeya is a complement factor D inhibitor and has a black box warning for serious infections caused by encapsulated bacteria. In addition, Voydeya is only available through a Risk Evaluation and Mitigation Strategy (REMS) program. ○ Voydeya has not been shown to be effective as monotherapy and should only be prescribed as an add-on to ravulizumab or eculizumab.

Approval Criteria

I. Initial Authorization

When a benefit, coverage of Voydeya may be approved when all of the following criteria are met **(A. through F.)**:

A. The member is 18 years of age or older.

B. Voydeya is being prescribed by or in consultation with a hematologist who specializes in the treatment of PNH.

C. The member has a diagnosis of PNH (ICD-10: D59.5).

D. The member has clinically significant extravascular hemolysis (EVH) demonstrated by one (1) of the following **(1. or 2.)**:

1. Hemoglobin ≤ 9.5 gm/dL

2. Absolute reticulocyte count (ARC) ≥ 120 x 10^9 /L

E. The member will use Voydeya in combination with ravulizumab or eculizumab for the treatment of PNH.

F. The member has been receiving ravulizumab or eculizumab for the treatment of PNH for ≥ 6 months.

II. Reauthorization

When a benefit, reauthorization of Voydeya may be approved when all of the following criteria are met **(A. and B.)**:

A. The member has experienced positive clinical response to therapy evidenced by one (1) of the following **(1. through 5.)**:

1. Increased or stabilization of hemoglobin (Hb) levels

2. Reduction in transfusions

3. Improvement in hemolysis

4. Decrease in LDH

5. Decreased reticulocyte count

B. The member continues to receive Voydeya in combination with ravulizumab or eculizumab.

III. An exception to some or all of the criteria above may be granted for select members and/or circumstances based on state and/or federal regulations.

Limitations of Coverage

I. Coverage of drug(s) addressed in this policy for disease states outside of the FDA-approved indications should be denied based on the lack of clinical data to support effectiveness and safety in other conditions unless otherwise noted in the approval criteria.

II. For Commercial or HCR members with a closed formulary, a non-formulary product will only be approved if the member meets the criteria for a formulary exception in addition to the criteria outlined within this policy.

Authorization Duration

Initial Authorization

- Commercial and HCR Plans: If approved, up to a 6 month authorization may be granted.

Reauthorization

- Commercial and HCR Plans: If approved, up to a 12 month authorization may be granted.

Automatic Approval Criteria

None

References:

1. Voydeya [package insert]. Boston, MA: Alexion Pharmaceuticals, Inc.; March 2024.
2. ASH Publications. When Does a PNH Clone Have Clinical Significance? Available at: https://www.ashpublications.org/hematology/article-pdf/2021/1/143/1852074/143babushok.pdf. Accessed April 4, 2025.
3. ICER. Iptacopan and Danicopan for Paroxysmal Nocturnal Hemoglobinuria. Available at: https://www.icer.org/wp-content/uploads/2023/07/PNH_Evidence-Report_For-Publication_02012024.pdf. Accessed April 4, 2025.
4. Parker CJ. Update on the Diagnosis and Management of Paroxysmal Nocturnal Hemoglobinuria. *Hematology Am Soc Hematol Educ Program*. 2016 Dec; 2016(1): 208-16.
5. AAMDS. Diseases and Treatments: Paroxysmal Nocturnal Hemoglobinuria (PNH). Available at: https://www.aamds.org/pnh. Accessed April 4, 2025.
6. UpToDate. Clinical Manifestations and Diagnosis of Paroxysmal Nocturnal Hemoglobinuria. Available at: https://www.uptodate.com. Accessed April 9, 2025.
7. NORD. Paroxysmal Nocturnal Hemoglobinuria. Available at: https://www.rarediseases.org/rare-diseases/paroxysmal-nocturnal-hemoglobinuria/. Accessed April 9, 2025.
8. National Institutes of Health. StatPearls [internet]. Paroxysmal Nocturnal Hemoglobinuria. Available at: https://www.ncbi.nlm.nih.gov/books/NBK562292/. Accessed April 9, 2025.
9. Bektas M, Copley-Merriman C, Khan S, et al. Paroxysmal Nocturnal Hemoglobinuria: Current Treatments and Unmet Needs. *J Manag Care Spec Pharm* 2020 Dec; 26(12-b Suppl).
10. AstraZeneca. Voydeya Approved in the US as Add-On Therapy to ravulizumab or eculizumab for Treatment of Extravascular Haemolysis in Adults with the Rare Disease PNH. Available at: https://www.astrazeneca.com/media-centre/press-releases/2024/voydeya-approved-in-us.html. Accessed April 9, 2025.
11. Drugs.com. Voydeya. Available at: https://www.drugs.com/voydeya.html. Accessed April 9, 2025.
12. Kulasekararaj A, Kuter D, Griffin M, et al. Biomarkers and laboratory assessments for monitoring the treatment of patients with paroxysmal nocturnal hemoglobinuria: Differences between terminal and proximal complement inhibition. *Blood Reviews. 2*023 May:59:101041. doi: 10.1016/j.blre.2023.101041. Epub 2023 Jan 14.