

## PHARMACY POLICY STATEMENT

### Indiana Medicaid

<b>DRUG NAME</b>	<b>Voydeya (danicopan)</b>
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Voydeya, approved by the FDA in 2024, is a small molecule complement factor D inhibitor indicated as add-on therapy to ravulizumab or eculizumab for the treatment of extravascular hemolysis (EVH) in adults with paroxysmal nocturnal hemoglobinuria (PNH).

PNH is a hematopoietic stem cell disorder in which activation of the complement system destroys red blood cells because of an acquired mutation in the *PIGA* gene. Common manifestations can include hemolytic anemia and fatigue. Thrombosis and bone marrow suppression may also occur.

C5 inhibitors greatly reduce intravascular hemolysis (IVH; occurring within blood vessels) and thrombosis, however, EVH can be a mechanistic consequence of therapy and may lead to remaining dependent on transfusions. Voydeya acts proximally in the alternative pathway of the complement cascade to control EVH while the co-administered C5 inhibitor maintains control over IVH.

Voydeya (danicopan) will be considered for coverage when the following criteria are met:

#### Paroxysmal Nocturnal Hemoglobinuria (PNH)

For **initial** authorization:

1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a hematologist; AND
3. Member has a documented diagnosis of PNH; AND
4. Member has been treated with a C5 inhibitor (e.g., ravulizumab or eculizumab) for at least 6 months and will continue; AND
5. Member has clinically evident EVH with both of the following:
  - a) Hemoglobin 9.5 g/dL or less, and
  - b) Absolute reticulocyte count (ARC)  $120 \times 10^9/L$  or greater; AND
6. Member has been or will be vaccinated against encapsulated bacteria (*Streptococcus pneumoniae*, *Neisseria meningitidis* types A, C, W, Y, and B).
7. **Dosage allowed/Quantity limit:**  
 Start 150 mg 3 times a day orally. If hemoglobin has not increased by greater than 2 g/dL after 4 weeks or a transfusion was required, may increase to 200 mg 3 times a day.  
 QL: 180 tablets per 30 days

***If all the above requirements are met, the medication will be approved for 6 months.***

For **reauthorization**:

1. Chart notes must show clinical evidence of positive response to therapy such as increased hemoglobin level, decreased need for transfusions, improved fatigue; AND
2. Member is continuing treatment with a C5 inhibitor and not using Voydeya as monotherapy.

***If all the above requirements are met, the medication will be approved for an additional 12 months.***

**CareSource considers Voydeya (danicopan) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.**

DATE	ACTION/DESCRIPTION
04/03/2024	New policy for Voydeya created.

References:

1. Voydeya [prescribing information]. Alexion Pharmaceuticals, Inc.; 2024.
2. Lee JW, Griffin M, Kim JS, et al. Addition of danicopan to ravulizumab or eculizumab in patients with paroxysmal nocturnal haemoglobinuria and clinically significant extravascular haemolysis (ALPHA): a double-blind, randomised, phase 3 trial. *Lancet Haematol.* 2023;10(12):e955-e965. doi:10.1016/S2352-3026(23)00315-0
3. Risitano AM, Marotta S, Ricci P, et al. Anti-complement Treatment for Paroxysmal Nocturnal Hemoglobinuria: Time for Proximal Complement Inhibition? A Position Paper From the SAAWP of the EBMT. *Front Immunol.* 2019;10:1157. Published 2019 Jun 14. doi:10.3389/fimmu.2019.01157
4. Oliver M, Patriquin CJ. Paroxysmal Nocturnal Hemoglobinuria: Current Management, Unmet Needs, and Recommendations. *J Blood Med.* 2023;14:613-628. Published 2023 Dec 6. doi:10.2147/JBM.S431493
5. Parker CJ. Update on the diagnosis and management of paroxysmal nocturnal hemoglobinuria. *Hematology Am Soc Hematol Educ Program.* 2016;2016(1):208-216. doi:10.1182/asheducation-2016.1.208
6. Patriquin CJ, Kiss T, Caplan S, et al. How we treat paroxysmal nocturnal hemoglobinuria: A consensus statement of the Canadian PNH Network and review of the national registry. *Eur J Haematol.* 2019;102(1):36-52. doi:10.1111/ejh.13176
7. Devos T, Meers S, Boeckx N, et al. Diagnosis and management of PNH: Review and recommendations from a Belgian expert panel. *Eur J Haematol.* 2018;101(6):737-749. doi:10.1111/ejh.13166
8. Bodó I, Amine I, Boban A, et al. Complement Inhibition in Paroxysmal Nocturnal Hemoglobinuria (PNH): A Systematic Review and Expert Opinion from Central Europe on Special Patient Populations. *Adv Ther.* 2023;40(6):2752-2772. doi:10.1007/s12325-023-02510-4
9. Sahin F, Akay OM, Ayer M, et al. Pegs PNH diagnosis, follow-up and treatment guidelines. *Am J Blood Res.* 2016;6(2):19-27. Published 2016 Aug 5.
10. Cançado RD, Araújo ADS, Sandes AF, et al. Consensus statement for diagnosis and treatment of paroxysmal nocturnal haemoglobinuria. *Hematol Transfus Cell Ther.* 2021;43(3):341-348. doi:10.1016/j.htct.2020.06.006

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Revised date: 04/03/2024