PRIOR AUTHORIZATION POLICY

POLICY: Complement Inhibitors – Voydeya Prior Authorization Policy
- Voydeya™ (danicopan tablets – Alexion)

REVIEW DATE: 05/02/2024; selected revision 05/22/2024

OVERVIEW

Voydeya, a complement Factor D inhibitor, is indicated as add-on therapy to Soliris® (eculizumab intravenous infusion) or Ultomiris® (ravulizumab-cwvz intravenous infusion or subcutaneous injection) for the treatment of extravascular hemolysis in adults with paroxysmal nocturnal hemoglobinuria (PNH).

Voydeya has a Boxed Warning about serious infections caused by encapsulated bacteria.[1] Voydeya is only available through a restricted access program, Voydeya Risk Evaluation and Mitigation Strategy (REMS).

Disease Overview

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, genetic disorder of hematopoietic stem cells.[2,3] The mutation in the X-linked gene phosphatidylinositol glycan class A (PIGA) results in a deficiency in the glycosylphosphatidylinositol (GPI) protein, which is responsible for anchoring other protein moieties to the surface of the erythrocytes. Loss of anchoring of these proteins causes cells to hemolyze and leads to complications such as hemolytic anemia, thrombosis, and peripheral blood cytopenias. PNH is a clinical diagnosis that should be confirmed with peripheral blood flow cytometry to detect the absence or severe deficiency of GPI-anchored proteins on at least two lineages.[2,5] Prior to the availability of complement inhibitors, only supportive management, in terms of managing the cytopenias and controlling thrombotic risk were available. Supportive measures include platelet transfusion, immunosuppressive therapy for patients with bone marrow failure, use of erythropoietin for anemias, and aggressive anticoagulation.

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Voydeya. All approvals are provided for the duration noted below. In cases where the approval is authorized in months, 1 month is equal to 30 days. Because of the specialized skills required for evaluation and diagnosis of patients treated with Voydeya as well as the monitoring required for adverse events and long-term efficacy, approval requires Voydeya to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Voydeya is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Paroxysmal Nocturnal Hemoglobinuria.** Approve for the duration noted if the patient meets ONE of the following (A or B):
   - **A)** Initial therapy. Approve for 3 months if the patient meets ALL of the following (i, ii, iii, and iv):
     - **i.** Patient is ≥ 18 years of age; AND
     - **ii.** Paroxysmal nocturnal hemoglobinuria diagnosis was confirmed by peripheral blood flow cytometry results showing the absence or deficiency of glycosylphosphatidylinositol-anchored proteins on at least two cell lineages; AND
     - **iii.** The medication is prescribed in combination with Soliris (eculizumab intravenous infusion) or Ultomiris (ravulizumab-cwvz intravenous infusion or subcutaneous injection); AND
     - **iv.** According to the prescriber, patient has clinically significant extravascular hemolysis (while receiving Soliris or Ultomiris), as evidenced by objective laboratory findings.; AND
       Note: Examples of objective laboratory findings include reduction in hemoglobin levels, elevated reticulocyte counts, increased transfusion requirements, transfusion-dependence.
     - **v.** The medication is prescribed by or in consultation with a hematologist.
   - **B)** Patient is Currently Receiving Voydeya. Approve for 1 year if the patient meets ALL of the following (i, ii, iii, and iv):
     - **i.** Patient is ≥ 18 years of age; AND
     - **ii.** The medication is prescribed in combination with Soliris (eculizumab intravenous infusion) or Ultomiris (ravulizumab-cwvz intravenous infusion or subcutaneous injection); AND
     - **iii.** According to the prescriber, patient is continuing to derive benefit from Voydeya; AND
       Note: Examples of benefit include increase in or stabilization of hemoglobin levels, decreased transfusion requirements or transfusion independence, reductions in hemolysis, improvement in Functional Assessment of Chronic Illness Therapy (FACIT)-Fatigue score.
     - **iv.** The medication is prescribed by or in consultation with a hematologist.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Voydeya is not recommended in the following situations:

1. **Concomitant Use with Empaveli (pegcetacoplan subcutaneous injection) or Fabhalta (iptacopan capsules).** There is no evidence to support concomitant use of Voydeya with Empaveli or Fabhalta.

2. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Voydeya tablets [prescribing information]. Boston, MA: Alexion; March 2024.
2. Cançado RD, da Silva Araújo A, Sandes AF, et al. Consensus statement for diagnosis and treatment of paroxysmal nocturnal haemoglobinuria. *Hematol Transfus Cell Ther*. 2021;43:341-348.
3. Shah N, Bhatt H. Paroxysmal Nocturnal Hemoglobinuria. [Updated 2023 Jul 31]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2023 Jan. Available from: https://www.ncbi.nlm.nih.gov/books/NBK562292/. Accessed on April 11, 2024.
4. Roth A, Maciejewski J, Nishinura JI, et al. Screening and diagnostic clinical algorithm for paroxysmal nocturnal hemoglobinuria: Expert consensus. *Eur J Haematol*. 2018;101(1):3-11.