PRIOR AUTHORIZATION POLICY

POLICY: Gaucher Disease – Substrate Reduction Therapy – Miglustat Prior Authorization Policy
- Zavesca® (miglustat capsules – Actelion, generic)

REVIEW DATE: 05/04/2022; selected revision 09/28/2022

OVERVIEW

Miglustat capsules (Zavesca, generic), a glucosylceramide synthase inhibitor, is indicated as monotherapy for the treatment of adults with mild to moderate **Gaucher disease type 1** for whom enzyme replacement therapy is not a therapeutic option (e.g., due to allergy, hypersensitivity, or poor venous access).[1]

Disease Overview

Gaucher disease is caused by a deficiency in the lysosomal enzyme β-glucocerebrosidase.[2] This enzyme is responsible for the breakdown of glucosylceramide into glucose and ceramide. In Gaucher disease, deficiency of the enzyme β-glucocerebrosidase results in the accumulation of glucosylceramide substrate in lysosomal compartment of macrophages, giving rise to foam cells or "Gaucher cells." Zavesca is a specific inhibitor of the enzyme glycosylceramide synthase, which is responsible for producing the substrate glucosylceramide.[1] By functioning as a substrate reduction therapy, Zavesca allows the residual activity of the deficient glucocerebrosidase enzyme to be more effective.

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of miglustat capsules (Zavesca, generic). All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with miglustat capsules as well as the monitoring required for adverse events and long-term efficacy, approval requires miglustat capsules to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of miglustat capsules (Zavesca, generic) is recommended in those who meet the following criteria:

FDA-Approved Indication

1. **Gaucher Disease Type 1.** Approve for 1 year if the patient meets the following criteria (A and B):
   - **A)** The diagnosis is established by one of the following (i or ii):
     - **i.** Demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts; OR
     - **ii.** Molecular genetic testing documenting glucocerebrosidase gene mutation; AND
   - **B)** The medication is prescribed by or in consultation with a geneticist, endocrinologist, metabolic disorder subspecialist, or a physician who specializes in the treatment of Gaucher disease or related disorders.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of miglustat capsules (Zavesca, generic) is not recommended in the following situations:

1. 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. Zavesca® capsules [prescribing information]. South San Francisco, CA: Actelion; October 2021.
2. Cerdelga® capsules [prescribing information]. Waterford, Ireland: Genzyme; July 2021.