

PRIOR AUTHORIZATION POLICY

POLICY: Gaucher Disease Substrate Reduction Therapy – Miglustat capsules (Zavesca® – Actelion Pharmaceuticals; generic)

DATE REVIEWED: 05/06/2020

OVERVIEW

Miglustat capsules (Zavesca), a glucosylceramide synthase inhibitor, is indicated as monotherapy for the treatment of adult patients 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 Generic miglustat is an AB-rated therapeutically equivalent generic of Zavesca.

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

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Zavesca/generic miglustat is recommended in those who meet the following criteria:

FDA-Approved Indications

- 1. Gaucher Disease Type I.** Approve for 1 year if Zavesca or generic miglustat is prescribed by or in consultation with a geneticist, endocrinologist, metabolic disorder sub-specialist, or a physician who specializes in the treatment of Gaucher Disease or related disorders.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Zavesca/generic miglustat has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions.

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

1. Zavesca[®] [prescribing information]. South San Francisco, CA: Actelion Pharmaceuticals US Inc.; February 2014.
2. Cerdelga[™] capsules [prescribing information]. Waterford, Ireland: Genzyme; August 2014.

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