Clinical Policy: Miglustat (Zavesca)

Reference Number: PA.CP.PHAR.164
Effective Date: 01/18
Last Review Date: 02/17

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for miglustat (Zavesca®).

Policy/Criteria
It is the policy of Pennsylvania Health and Wellness that Zavesca is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Type 1 Gaucher Disease (must meet all):

1. Diagnosis of mild to moderate Type 1 Gaucher Disease (GD1) confirmed by one of the following:
   a. Enzyme assay demonstrating a deficiency in beta-glucocerebrosidase activity;
   b. DNA testing;
2. Member has failed at least two enzyme replacement therapies (i.e., Cerezyme [imiglucerase], Elelyso [taliglucerase alfa], VPRIV [velaglucerase alfa]) or is unable to take enzyme replacement therapies due to one of the following:
   a. Allergy or hypersensitivity;
   b. Poor venous access;
3. Zavesca is prescribed as monotherapy;
4. Prescribed daily dose of Zavesca does not exceed 300 mg;
5. Member does not have severe renal impairment (i.e., CrCl <30 mL/min/1.73 m^2).

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Type 1 Gaucher Disease (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy;
3. Zavesca is prescribed as monotherapy;
4. Prescribed daily dose of Zavesca does not exceed 300 mg;
5. Member has none of the following reasons to discontinue Zavesca therapy:
   a. Severe renal impairment (i.e., CrCl <30 mL/min/1.73 m^2);
   b. Unresolved hand tremors despite dose reduction.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or
2. Refer to PA.CP.PHAR.57 - Global Biopharm Policy**.**

Background

Description/Mechanism of Action:

Type 1 Gaucher disease is caused by a functional deficiency of glucocerebrosidase, the enzyme that mediates the degradation of the glycosphingolipid glucosylceramide. Miglustat functions as a competitive and reversible inhibitor of the enzyme glucosylceramide synthase, the initial enzyme in a series of reactions which results in the synthesis of most glycosphingolipids. Zavesca helps reduce the rate of glycosphingolipid biosynthesis so that the amount of glycosphingolipid substrate is reduced to a level which allows the residual activity of the deficient glucocerebrosidase enzyme to be more effective (substrate reduction therapy). In vitro and in vivo studies have shown that miglustat can reduce the synthesis of glucosylceramide-based glycosphingolipids.

Formulations:

Zavesca (miglustat): Capsules for oral use

- 100 mg/capsule

FDA Approved Indications:

Zavesca is a glucosylceramide synthase inhibitor/oral capsule formulation indicated as monotherapy for the treatment of:

- Adult patients with mild to moderate Type 1 Gaucher disease for whom enzyme replacement therapy is not a therapeutic option (e.g. due to allergy, hypersensitivity, or poor venous access).

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
N/A	

Reviews, Revisions, and Approvals	Date	Approval Date

References

1. Zavesca prescribing information. Irvine, CA: Actelion Pharmaceuticals US, Inc.; February 2016. Available at https://www.zavesca.com/pdf/ZAVESCA-Full-Prescribing-Information.pdf. Accessed December 21, 2016.
2. Charrow J, Andersson HC, Kaplan P. Enzyme replacement therapy and monitoring for children with Type 1 Gaucher disease: Consensus recommendations. *J Pediatr.* 2004; 144: 112-20.
3. Hollak, CEM, Weinreb NJ. The attenuated/late onset lysosomal storage disorders: Therapeutic goals and indications for enzyme replacement treatment in Gaucher and Fabry disease. *Best Pract Res Clin Endocrinol Metab.* 2015; 29: 205-218.