PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Zavesca (miglustat)
BILLING CODE	Must use valid NDC
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

Zavesca is a substrate reduction therapy, FDA approved in 2003 for the treatment of Gaucher disease type 1. Gaucher disease is a rare, inherited, lysosomal storage disorder. In Gaucher disease, mutations of the GBA gene cause deficiency of the enzyme glucocerebrosidase (acid beta-glucosidase), resulting in the accumulation of glucocerebroside (glucosylceramide [GLC]) in the lysosomes of macrophages to form "Gaucher cells," especially in the bone marrow, spleen, and liver. Prominent symptt.4 (i-(m)3.4 es)-1.1 nt-(ff)3.4 n aa(r)0.6 (v)3.8 oausym coter to(s)3.7 t ofecyme
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Zavesca (miglustat) will be considered for coverage when the following criteria are met:

For **reauthorization**:

1. Chart notes must document improvement in one or more of the following parameters compared to baseline:
   a) Hemoglobin level
   b) Platelet count
   c) Spleen and/or liver volumes
   d) Bone outcomes

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

CareSource considers Zavesca (miglustat) 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
06/29/2017	New policy for Zavesca created.
08/06/2021	Transferred to new template. Added references. Added specialist requirement. Elaborated on diagnostic requirement. Removed restriction of ERT within last 6 months. Removed baseline measures requirement. Added that they must present with symptoms. Changed renewal criteria. Changed approval durations from 6 months to 12 months.

References:

1. Zavesca [package insert]. South San Francisco, CA; Actelion Pharmaceuticals US, Inc: 1/2021.
2. Cox T, Lachmann R, Hollak C, et al. Novel oral treatment of Gaucher's disease with N-butyldeoxynojirimycin (OGT 918) to decrease substrate biosynthesis. *Lancet*. 2000;355(9214):1481-1485. doi:10.1016/S0140-6736(00)02161-9
3. Elstein D, Hollak C, Aerts JM, et al. Sustained therapeutic effects of oral miglustat (Zavesca, N-butyldeoxynojirimycin, OGT 918) in type I Gaucher disease. *J Inherit Metab Dis*. 2004;27(6):757-766. doi:10.1023/B:BOLI.0000045756.54006.17
4. Cox TM, Aerts JM, Andria G, et al. The role of the iminosugar N-butyldeoxynojirimycin (miglustat) in the management of type I (non-neuronopathic) Gaucher disease: a position statement. *J Inherit Metab Dis*. 2003;26(6):513-526. doi:10.1023/a:1025902113005
5. Elstein D, Dweck A, Attias D, et al. Oral maintenance clinical trial with miglustat for type I Gaucher disease: switch from or combination with intravenous enzyme replacement. *Blood*. 2007;110(7):2296-2301. doi:10.1182/blood-2007-02-075960
6. Biegstraaten M, van Schaik IN, Aerts JM, Hollak CE. 'Non-neuronopathic' Gaucher disease reconsidered. Prevalence of neurological manifestations in a Dutch cohort of type I Gaucher disease patients and a systematic review of the literature. *J Inherit Metab Dis*. 2008;31(3):337-349. doi:10.1007/s10545-008-0832-y

Effective date: 01/01/2022
Revised date: 08/06/2021