## SPECIALTY GUIDELINE MANAGEMENT

# ZAVESCA (miglustat) miglustat (generic)

#### **POLICY**

#### I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

### A. FDA-Approved Indications

Zavesca is 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).

#### B. Compendial Uses

Niemann-Pick disease, type C

All other indications are considered experimental/investigational and not medically necessary.

#### **II. DOCUMENTATION**

Submission of the following information is necessary to initiate the prior authorization review:

- A. Gaucher disease type 1: beta-glucocerebrosidase enzyme assay or genetic testing results supporting diagnosis
- B. Niemann-Pick disease, type C: genetic testing results showing mutations in NPC1 or NPC2 genes.

#### III. CRITERIA FOR INITIAL APPROVAL

## A. Gaucher disease type 1

Authorization of 12 months may be granted for treatment of Gaucher disease type 1 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing, and the member has a documented inadequate response or intolerable adverse events with enzyme replacement therapy.

#### B. Niemann-Pick disease, type C

Authorization of 12 months may be granted for treatment of Niemann-Pick disease, type C when the diagnosis was confirmed by genetic testing results showing mutations in *NPC1* or *NPC2* genes.

#### IV. CONTINUATION OF THERAPY

#### A. Gaucher disease type 1

miglustat-Zavesca 2098-A SGM P2021.docx

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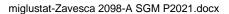
Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for Gaucher disease type 1 who are not experiencing an inadequate response or any intolerable adverse events from therapy.

### B. Niemann-Pick disease, type C

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for Niemann-Pick disease, type C who are not experiencing an inadequate response or any intolerable adverse events from therapy.

#### V. REFERENCES

- 1. Zavesca [package insert]. South San Francisco, CA: Actelion Pharmaceuticals US, Inc.; December 2020.
- 2. miglustat [package insert]. Horsham, PA: Patriot Pharmaceuticals, LLC.; November 2017.
- 3. American Society of Health System Pharmacists. AHFS Drug Information. Bethesda, MD. Electronic version, 2021. Available with subscription. URL: <a href="http://online.lexi.com/crlsql/servlet/crlonline">http://online.lexi.com/crlsql/servlet/crlonline</a>. Accessed January 28, 2021.
- 4. DRUGDEX System (electronic version). Micromedex Truven Health Analytics. Available with subscription. URL: www.micromedexsolutions.com. Accessed January 28, 2021.
- 5. National Organization for Rare Disorders. (2003). NORD guide to rare disorders. Philadelphia: Lippincott Williams & Wilkins.



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