Reference number
2098-A

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

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: http://online.lexi.com/crlsql/servlet/crlonline. 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.