

Market Applicability														
Market	DC	FL & FHK	FL MMA	FL LTC	GA	KS	KY	MD	NJ	NV	NY	TN	TX	WA
Applicable	X	X	NA	NA	X	NA	X	X	X	X	X	NA	NA	NA

*FHK- Florida Healthy Kids

Zavesca (miglustat)

Override(s)	Approval Duration
Prior Authorization	1 year

Medications
Zavesca (miglustat) 100mg capsules

APPROVAL CRITERIA

- I. Individual is 18 years of age or older; **AND**
- II. Individual has a diagnosis of type 1 Gaucher disease confirmed by either of the following (Weinreb et al. 2004, Wang et al. 2011):
 - A. Glucocerebrosidase activity less than or equal to 30% of normal activity in the white blood cells or skin fibroblasts; **OR**
 - B. Genotype testing indicates mutation of two alleles of the glucocerebrosidase genome;

AND

- III. There are clinically significant manifestations of Gaucher disease, including **any** of the following:
 - A. Skeletal disease as demonstrated by radiologic evidence of **any** of the following:
 1. Avascular necrosis; **OR**
 2. Erlenmeyer flask deformity (failure of bone remodeling); **OR**
 3. Lytic disease; **OR**
 4. Marrow infiltration; **OR**
 5. Osteopenia; **OR**
 6. Osteosclerosis; **OR**
 7. Pathological fracture; **OR**
 8. Joint deterioration or replacement;
 - OR**
 - B. Presents with **at least two** of the following (Weinreb et al. 2004, Mistry et al. 2015):
 1. Clinically significant hepatomegaly as confirmed by medical imaging [such as but not limited to, volumetric magnetic resonance imaging (MRI)]; **OR**
 2. Clinically significant splenomegaly as confirmed by medical imaging (such as but not limited to, volumetric MRI); **OR**

This policy does not apply to health plans or member categories that do not have pharmacy benefits, nor does it apply to Medicare. Note that market specific restrictions or transition-of-care benefit limitations may apply.

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3. Hemoglobin less than or equal to 11.5 g/dL for females and less than 12.5 g/dL for males, or 1.0g/dL below lower limit for normal for age and sex; **OR**
4. Platelet count less than or equal to 120,000mm³

AND

- IV. Enzyme replacement therapy (for example, Cerezyme (imiglucerase), Elelyso (taliglucerase alfa), VPRIV (velaglucerase alfa)) is not a therapeutic option, for reasons such as but not limited to any of the following (Label, Weinreb et al. 2005):
- A. Medically unmanageable hypersensitivity; **OR**
 - B. Development of therapy-limiting inhibitory antibodies; **OR**
 - C. Poor peripheral or central venous access.

Zavesca (miglustat) may **not** be approved for the following:

- I. Severe type 1 Gaucher disease (hemoglobin less than 9 g/dL, platelet count less than 50,000 mm³, or those at risk of developing new bone complications) (Weinreb et al. 2005); **OR**
- II. Individual has severe renal impairment (less than 30 mL/min/1.73 m²); **OR**
- III. Individual has mild, moderate, or severe hepatic impairment or cirrhosis; **OR**
- IV. When given in conjunction with any of the following:
 - A. Cerdelga (eliglustat); **OR**
 - B. Gaucher disease enzyme replacement therapies [Cerezyme (imiglucerase), Elelyso (taliglucerase alfa), or VPRIV (velaglucerase alfa)].

State Specific Mandates		
State name	Date effective	Mandate details (including specific bill if applicable)
N/A	N/A	N/A

Key References:

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Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2018. URL: <http://www.clinicalpharmacology.com>. Updated periodically.

DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. <http://dailymed.nlm.nih.gov/dailymed/about.cfm>. Accessed: April 10, 2018

DrugPoints® System (electronic version). Truven Health Analytics, Greenwood Village, CO. Updated periodically.

Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2018; Updated periodically.

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