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	X

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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**

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	X

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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 120,000mm^3

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 mm^3, 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 m^2); **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:

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	X

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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.

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.