Cerdelga (eliglustat)

Override(s)	Approval Duration
Prior Authorization Quantity Limit	1 year

Medications	Quantity Limit
Cerdelga (eliglustat)	May be subject to quantity limit

APPROVAL CRITERIA

Requests for Cerdelga (eliglustat) may be approved when the criteria below are met:

I. Individual is 18 years of age or older; **AND**

II. Documentation is provided that individual has a diagnosis of Type 1 Gaucher disease confirmed by either of the following (Wang, 2011; Weinreb, 2004):
   A. Deficiency in glucocerebrosidase enzyme activity as measured in the white blood cells or skin fibroblasts; **OR**
   B. Genotype testing indicates mutation of two alleles of the glucocerebrosidase genome;

AND

III. Documentation is provided that individual has clinically significant manifestations of Gaucher disease including (Andersson, 2005; Weinreb, 2004):
   A. Skeletal disease (including, but not limited to avascular necrosis, Erlenmeyer flask deformity, osteopenia or pathological fracture);
   OR
   B. Presents with at least two of the following:
      1. Clinically significant splenomegaly; **OR**
      2. Clinically significant hepatomegaly; **OR**
      3. Hemoglobin at least 1.0 g/dL below lower limit for normal for age and sex; **OR**
      4. Platelet count less than or equal to 120,000 mm^3;

AND

IV. Individual is a CYP2D6 extensive metabolizer (EM), intermediate metabolizer (IM), or poor metabolizer (PM) as confirmed by a FDA-approved genotype test.

Continuation requests for Cerdelga (eliglustat) may be approved if the following criterion is met:

I. There is confirmation of clinically significant improvement in clinical signs and symptoms of disease (including but not limited to reduction of spleen volume, reduction of liver

volume, resolution of anemia, resolution of thrombocytopenia, reduction in fatigue, improvement in skeletal manifestations).

Cerdelga (eliglustat) may **not** be approved for the following:

I. Individual has any of the following diagnoses:

   A. End-stage renal disease (ESRD) in CYP2D6 EM individuals; **OR**
   B. Mild, moderate or severe renal impairment or end-stage renal disease (ESRD) in CYP2D6 IM or PM individuals; **OR**
   C. Moderate or severe hepatic impairment in CYP2D6 EM individuals; **OR**
   D. Mild, moderate or severe hepatic impairment in CYP2D6 IM or PM individuals; **OR**
   E. Pre-existing cardiac disease or long QT syndrome;

OR

II. When given in conjunction with any of the following:
   A. Zavesca (miglustat); **OR**
   B. Gaucher disease enzyme replacement therapies [Cerezyme (imiglucerase), Elelyso (taliglucerase alfa), or VPRIV (velaglucerase alfa)]; **OR**
   C. Class IA (for example, quinidine, procainamide) or Class III (for example, amiodarone, sotalol) antiarrhythmic; **OR**
   D. Moderate or strong CYP2D6 inhibitor (for example, paroxetine, terbinafine) with a moderate or strong CYP3A inhibitor (for example, ketoconazole) in CYP2D6 EM or IM individuals; **OR**
   E. Moderate or strong CYP3A inhibitor (for example, ketoconazole, fluconazole) in CYP2D6 IM individuals; **OR**
   F. Weak, moderate, or strong CYP3A inhibitor (for example, ranitidine, ketoconazole, fluconazole) in CYP2D6 PM individuals; **OR**
   G. Strong CYP3A inducers (for example, rifampin, phenytoin) in CYP2D6 EM, IM, or PM individuals; **OR**
   H. Moderate or strong CYP2D6 inhibitor in CYP2D6 EM individuals with mild hepatic impairment;

OR

III. Individuals who are CYP2D6 ultra-rapid or indeterminate metabolizers.

Key References:

1. Andersson HC, Charrow J, Kaplan P, et al., International Collaborative Gaucher Group (ICGG) US Regional Coordinators. Individualization of long term enzyme replacement (ERT) for Gaucher's disease. *Genet Med*. 2005; 7(2):105-110.
2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: June 12, 2021.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Grabowski GA, Barton NW, Pastores G, et al. Enzyme therapy in type 1 Gaucher disease: comparative efficacy of mannose-terminated glucocerebrosidase from natural and recombinant sources. *Ann Intern Med*. 1995;122:33-39.
5. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2021; Updated periodically.
6. Mistry PK, Cappellini MD, Lukina E, et al. A reappraisal of Gaucher disease – Diagnosis and disease management algorithms. *Am J Hematol*. 2011; 86(1):110-115.
7. Turkia HB, Gonzalez DE, Barton NW, et al. Velaglucerase alfa enzyme replacement therapy compared with imiglucerase in patients with Gaucher disease. *Am J Hematol.* 2013; 88(3):179-84.
8. Vellodi A, Tylki-Szymanska A, Davies EH, et al. Management of neuropathic Gaucher disease: revised recommendations. J Inherit Metab Dis. 2009; 32(5):660-664.
9. Wang RY, Bodamer OA, Watson MS, Wilcox WR; American College of Medical Genetics (ACMG) Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genet Med*. 2011; 13(5):457-484.
10. Weinreb NJ, Aggio MC, Andersson HC, et al. Gaucher disease type 1: Revised recommendations on evaluations and monitoring for adult patients. *Semin Hematol*. 2004; 41(Suppl 5):15-22.
11. Zimran A, Brill-Almon E, Chertkoff R, et al. Pivotal trial with plant cell-expressed recombinant glucocerebrosidase, taliglucerase alfa, a novel enzyme replacement therapy for Gaucher disease. *Blood.* 2011; 118: 5767-5773.