

## Substrate Reduction Therapy Prior Authorization

<b>Drug(s) Applied:</b>	<b>Cerdelga (eliglustat), miglustat, Yargesa (miglustat)</b>
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### Criteria:

Drug(s) Applied will be approved when the requested medication is being used for an FDA approved indication and all of the following criteria are met:

#### I. Initial Therapy Criteria

##### A. Gaucher disease type 1 (GD1) as indicated by chart notes within past 90 days

##### 1. Diagnosis confirmed by ONE of the following:

- Patient has baseline (prior to therapy) acid  $\beta$ -glucocerebrosidase (GCase) enzyme activity of less than or equal to 15% of mean normal in fibroblasts, leukocytes, or other nucleated cells **or**
- Genetic analysis confirmed by two (2) pathogenic variants in the glucocerebrosidase (GBA1) gene **and**

##### 2. Patient does NOT have neuronopathic symptoms indicative of Gaucher disease type 2 or type 3 [e.g., bulbar signs (e.g., stridor, strabismus, swallowing difficulty), pyramidal signs (e.g., opisthotonos, head retroflexion, spasticity, trismus), oculomotor apraxia, tonic-clonic seizures, myoclonic epilepsy, dementia, ataxia] **and**

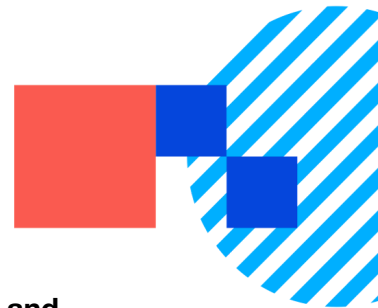
##### 3. At least two (2) of the following clinical presentations are present at baseline:

- Anemia defined as mean hemoglobin (Hb) level below the testing laboratory's lower limit of the normal range based on age and gender **or**
- Thrombocytopenia (platelet count < 120,000/microliter on at least 2 measurements) **or**
- Hepatomegaly **or**
- Splenomegaly or prior splenectomy **or**
- Growth failure (i.e., height < 2.5 percentile or delayed bone age) **or**
- Bone crisis or bone disease, including but not limited to osteopenia, marrow infiltration, pathologic fractures, or Erlenmeyer's flask deformity, with other causes ruled out **and**

##### 4. Prescriber has assessed baseline status of hemoglobin level, platelet count, liver volume, and spleen volume **and**

##### 5. If for Cerdelga, BOTH of the following:

- Patient is a CYP2D6 extensive metabolizer (EM), intermediate metabolizer (IM), or poor metabolizer (PM), as detected by an



- FDA-cleared test for determining CYP2D6 genotype **and**
- b) Patient has documented failure to miglustat **and**
- 6. Patient's age is within FDA labeling for the requested indication or there is support for using the requested agent for the patient's age for the requested indication **and**
- 7. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., endocrinology, genetics) **and**
- 8. Patient will NOT be using the requested agent in combination with another substrate reduction therapy agent (e.g., Cerdelga, Zavesca) for the requested indication **and**
- 9. Chart notes and/or prescriber do not provide documentation of any FDA labeled contraindications or significant warnings and precautions to the requested drug, or prescriber has documented that the benefits outweigh the risk
  - a) If for Cerdelga, contraindications/precautions include but are not limited to hepatic impairment or renal impairment, both depending on CYP2D6 metabolizer status, and pre-existing cardiac disease, arrhythmias, and long QT syndrome

**Approval Duration:** 12 months

## II. Continued Therapy Criteria

- A.** Gaucher disease type 1 (GD1) as indicated by chart notes within past 12 months
  - 1. Patient has been previously approved for the requested agent through the plan's Prior Authorization process or meets the initial therapy criteria above **and**
  - 2. Documented clinical benefit since starting the requested agent as indicated by improvement or stabilization of at least two (2) of the following:
    - a) Hemoglobin level > 11.0g/dL for women and children and > 12.0g/dL for men within 12-24 months of treatment or
    - b) Increased platelet count (sufficient to decrease the risk of bleeding) within 12 months of treatment or
    - c) Decreased hepatic volume or
    - d) Decreased spleen volume within 12-24 months of treatment or
    - e) Normalized growth as indicated by height at target within 24 months of treatment or
    - f) Reduction or remission of bone pain or crisis **and**
  - 3. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., endocrinology, genetics) **and**
  - 4. Patient will NOT be using the requested agent in combination with another substrate reduction therapy agent (e.g., Cerdelga, Zavesca) for the requested indication **and**



5. Chart notes and/or prescriber do not provide documentation of any FDA labeled contraindications or significant warnings and precautions to the requested drug, or prescriber has documented that the benefits outweigh the risk
  - a. If for Cerdelga, contraindications/precautions include but are not limited to hepatic impairment or renal impairment, both depending on CYP2D6 metabolizer status, and pre-existing cardiac disease, arrhythmias, and long QT syndrome

**Approval Duration:** 12 months

**Policy Owned by:** Curative PBM team