curative

Substrate Reduction Therapy Prior Authorization

Drug(s) Applied:	**miglustat, Yargesa** (miglustat)

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:
     a) Patient has baseline (prior to therapy) acid β-glucocerebrosidase (GCase) enzyme activity of less than or equal to 15% of mean normal in fibroblasts, leukocytes, or other nucleated cells **or**
     b) 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:
     a) Anemia defined as mean hemoglobin (Hb) level below the testing laboratory's lower limit of the normal range based on age and gender or
     b) Thrombocytopenia (platelet count < 120,000/microliter on at least 2 measurements) or
     c) Hepatomegaly or
     d) Splenomegaly or prior splenectomy or
     e) Growth failure (i.e., height < 2.5 percentile or delayed bone age) or
     f) 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. 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**

6. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., endocrinology, genetics) **and**
7. Patient will NOT be using the requested agent in combination with another substrate reduction therapy agent (e.g., Cerdelga, Zavesca) for the requested indication

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

Approval Duration: 12 months

Policy Owned by: Curative PBM team