Enzyme Replacement Therapy for Gaucher Disease

Override(s) | Approval Duration
---|---
Prior Authorization | 1 Year

Medications
Cerezyme (imiglucerase)
ELELYSO (taliglucerase alfa)
VPRIV (velaglucerase alfa)

**APPROVAL CRITERIA**

Requests for enzyme replacement therapy for Gaucher disease [Cerezyme (imiglucerase), Eleyso (taliglucerase) and Vpriv (velaglucerase)] may be approved if the following criteria are met:

I. Individual is 18 years of age and older with a diagnosis of type 1 Gaucher disease and the following criteria are met:
   A. Type 1 Gaucher disease is confirmed by either (Weinreb, 2004; Wang, 2011):
      1. Deficiency in glucocerebrosidase enzyme activity as measured in the white blood cells or skin fibroblasts; OR
      2. Genotype testing indicates mutation of two alleles of the glucocerebrosidase genome;
   AND
   B. Individual has clinically significant manifestations of Gaucher disease including (Andersson, 2005; Weinreb, 2004):
      1. Skeletal disease (such as but not limited to avascular necrosis, Erlenmeyer flask deformity, osteopenia or pathological fracture); OR
      2. Two or more of the following:
         a. Clinically significant hepatomegaly; OR
         b. Clinically significant splenomegaly; OR
         c. Hemoglobin at least 1.0g/dL below lower limit for normal for age and sex; OR
         d. Platelet count less than or equal to 120,000mm³;

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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OR

II. Individual is less than 18 years of age with a diagnosis of type 1 Gaucher disease and the following criteria are met:
   A. Type 1 Gaucher disease is confirmed by either (Kaplan, 2013; Wang, 2011):
      1. Deficiency in glucocerebrosidase activity as measured in the white blood cells or skin fibroblasts; OR
      2. Genotype testing indicates mutation of two alleles of the glucocerebrosidase genome;
   AND
   B. Individual has clinically significant manifestations of Gaucher disease (such as but not or growth failure) (Andersson, 2005);

OR

III. Individual is 18 years of age or older with a diagnosis of type 3 Gaucher disease and the following criteria are met (Kaplan, 2013):
   A. Type 3 Gaucher disease is confirmed by genotype testing indicating mutation of two alleles of the glucocerebrosidase genome (Kaplan, 2013; Wang, 2011); **AND**
   B. Individual has clinically significant manifestations of Gaucher disease including (Andersson, 2005; Weinreb, 2004):
      1. Skeletal disease (such as but not limited to avascular necrosis, Erlenmeyer flask deformity, osteopenia or pathological fracture); OR
      2. Two or more of the following:
         a. Clinically significant hepatomegaly; OR
         b. Clinically significant splenomegaly; OR
         c. Hemoglobin at least 1.0 g/dL below lower limit for normal for age and sex); OR
      d. Platelet count less than or equal to 120,000mm$^3$;
   **AND**
   C. There are neurological findings consistent with type 3 Gaucher disease based on neurological evaluation including brain imaging [magnetic resonance imaging (MRI) or computed tomography (CT)] and electroencephalography (EEG) (Vellodi, 2009);

OR

IV. Individual is less than 18 years of age with type 3 Gaucher disease and the following criteria are met (Kaplan, 2013):
   A. Type 3 Gaucher disease is confirmed by genotype testing indicating mutation of two alleles of the glucocerebrosidase genome (Kaplan, 2013; Wang, 2011); **AND**
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 |

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B. Individual has clinically significant manifestations of Gaucher disease (such as but not limited to hepatomegaly, splenomegaly, anemia, thrombocytopenia, skeletal disease or growth failure) (Andersson, 2005);

AND

C. There are neurological findings consistent with type 3 Gaucher disease based on neurological evaluation including brain imaging [magnetic resonance imaging (MRI) or computed tomography (CT)] and electroencephalography (EEG) (Vellodi, 2009).

Enzyme replacement therapy for Gaucher disease [Cerezyme (imiglucerase), Elelyso (taliglucerase) and Vpriv (velaglucerase)] may not be approved for the following:

I. Individuals with type 2 Gaucher disease; OR

II. Use in conjunction with another enzyme replacement therapy agent or substrate reduction therapy agent [Cerdelga (eliglustat), Zavesca (miglustat)] for the treatment of Gaucher disease.

State Specific Mandates

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<tr>
<th>State name</th>
<th>Date effective</th>
<th>Mandate details (including specific bill if applicable)</th>
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<tbody>
<tr>
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Key References:

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

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### Market Applicability

| Market | DC | FL & FHA | 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 | X | NA | NA | NA |

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