Pharmacy Medical Necessity Guidelines: Cerezyme® (imiglucerase), Elelyso® (taliglucerase alfa) and VPRIV® (velaglucerase alfa)

Effective: July 11, 2017

Prior Authorization Required: √
Type of Review – Care Management
Not Covered: 
Type of Review – Clinical Review: √
Pharmacy (RX) or Medical (MED) Benefit: MED
Department to Review: PRECERT/MM

This Pharmacy Medical Necessity Guideline applies to the following:

Tufts Health Plan Commercial Plans
☑ Tufts Health Plan Commercial Plans – large group plans
☑ Tufts Health Plan Commercial Plans – small group and individual plans

Tufts Health Public Plans
☑ Tufts Health Direct – Health Connector
☑ Tufts Health Together – A MassHealth Plan
☑ Tufts Health RITogether – A Rite Care + Rhody Health Partners Plan

Tufts Health Freedom Plan products
☑ Tufts Health Freedom Plan – large group plans
☑ Tufts Health Freedom Plan – small group plans

Fax Numbers:
All plans except Tufts Health Public Plans:
PRECERT: 617.972.9409
Tufts Health Public Plans:
MM: 888.415.9055

Note: For Tufts Health Plan Medicare Preferred Members, please refer to the Tufts Health Plan Medicare Preferred Prior Authorization Criteria. Background, applicable product and disclaimer information can be found on the last page.

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS
Cerezyme (imiglucerase for injection) is indicated for long-term enzyme replacement therapy for pediatric and adult patients with a confirmed diagnosis of Type 1 Gaucher disease that results in one or more of the following conditions: anemia, thrombocytopenia, bone disease, or hepatomegaly or splenomegaly.

Elelyso (taliglucerase alfa) for injection is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for the treatment of pediatric and adult patients with a confirmed diagnosis of Type 1 Gaucher disease.

VPRIV (velaglucerase alfa) is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy for pediatric and adult patients with type 1 Gaucher disease.

Gaucher disease is a rare and debilitating genetic disorder in which patients lack the enzyme b-glucocerebrosidase, which is essential for the proper lipid metabolism. As a result of this missing enzyme, there is a build-up of the glycolipid glucocerebroside, which can cause a host of problems, most importantly, hepatomegaly (enlarged liver), splenomegaly (enlarged spleen), bone disease and severe anemia (low blood counts). The mainstay of treatment for this disease focuses on replacing the missing enzyme, which provides some relief, but is not a cure.

COVERAGE GUIDELINES
The plan may authorize coverage of Cerezyme (imiglucerase), Elelyso (taliglucerase alfa) or VPRIV (velaglucerase alfa) for Members when the following criteria are met:

Members 18 Years of Age or Over
1. Documented Diagnosis of Type 1 Gaucher disease by a specialist AND
2. Documentation of one of the following:
   a. Has symptomatic manifestations of skeletal disease as confirmed by radiological assay, including:
      i. Joint deterioration
      ii. Pathological fracture
      iii. Avascular fracture
      iv. Definite Osteopenia
      v. Marrow infiltration
   b. Presents with two or more of the following:
i. Anemia ≤12.5g/dl for males and ≤11.5g/dl for females
ii. Platelet count ≤120,000/mm³
iii. Hepatomegaly, defined as liver volume 25% greater than normal
iv. Splenomegaly, defined as spleen volume 5 times normal or greater

**Member Under 18 Years of Age**
1. Documented diagnosis of Type 1 Gaucher disease by a pediatric metabolic specialist
   **AND**
2. Documentation of one of the following:
   a. Bone pain
   b. Abdominal pain
   c. Fatigue
   d. Exertional limitations
   e. Weakness
   f. Cachexia
   g. Hepatosplenomegaly
   h. Thrombocytopenia, defined as platelet count <60,000 mm³
   i. Anemia, defined as hemoglobin < 2 g/dl below their lower limit of normal for age and sex
   j. Growth failure secondary to Gaucher Disease
   k. Evidence of skeletal involvement as confirmed by radiographic assay to include:
      i. Erlenmeyer Flask Deformity (EFD)
      ii. Avascular Necrosis of bone
      iii. Destructive lesions of bone

**Dosing Recommendations**
Symptomatic Members may receive doses up to, but not exceeding, 60 units/kg infused every 2 weeks.

**LIMITATIONS**
1. The plan does NOT cover enzyme replacement therapy for Type 2 or Type 3 Gaucher Disease.

**CODES**
The following HCPCS/CPT code(s) are:

<table>
<thead>
<tr>
<th>Code</th>
<th>Description</th>
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<tbody>
<tr>
<td>J1786</td>
<td>Injection, imiglucerase, 10 units</td>
</tr>
<tr>
<td>J3060</td>
<td>Injection, taliglucerase alfa, 10 units</td>
</tr>
<tr>
<td>J3385</td>
<td>Injection, velaglucerase alfa, 100 units</td>
</tr>
</tbody>
</table>

**REFERENCES**
6. Cox TM, Drellichman G, Cravo R et al. ENCORE: A multi-national, randomized, controlled, open-label, non-inferiority study comparing eliglustat with imiglucerase in Gaucher disease type 1 patients on enzyme replacement therapy who have reached therapeutic goals. Poster presented at Lysosomal Disease Network World Symposium. San Diego, CA; 2014 February 12.


**APPROVAL HISTORY**

September 2001: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

- **December 14, 2004:** Remove Type 3 Gaucher disease from Coverage Criteria. Add Type 3 to Coverage Limitations.
- **November 8, 2005:** No changes
- **September 12, 2006:** Added generic descriptors for both Ceredase (algglucerase) or Cerezyme (imiglucerase) under I. Clinical Coverage Criteria.
- **September 11, 2007:** No changes
- **May 13, 2008:** Changed criteria #1 for Children under the age of 18 to read as, "Documented diagnosis by a pediatric metabolic specialist of Type 1 Gaucher disease and one of the following:" Added "hepatosplenomegaly to coverage criteria #1 for Children under the age of 18. Removed statement ("And one or more of the following") between coverage criteria #1 and #2 for Children under the age of 18. Removed Dosing Criteria section and changed dose recommendation statement for children to read as, “Dosing Recommendations: Symptomatic members may receive doses up to, but not exceeding, 60units/kg infused every 2 weeks.”
- **May 12, 2009:** No changes
- **January 1, 2010:** Removal of Tufts Health Plan Medicare Preferred language (separate criteria have been created specifically for Tufts Health Plan Medicare Preferred).
- **May 11, 2010:** No changes
- **July 13, 2010:** Added VPRIV (velaglucerase alfa) to pharmacy medical necessity guidelines.
- **January 1, 2011:** Administrative Update: Added reimbursement codes J1786 and J3385.
- **July 12, 2011:** No changes
- **May 8, 2012:** Administrative Update: Removed reimbursement codes J0205 and J1785. Removed Ceredase from Medical Necessity Guidelines, product is no longer available.
- **September 11, 2012:** Added Elelyso (taliglucerase alfa) to the Medical Necessity Guideline.
- **January 1, 2013:** Administrative Update: Added reimbursement code C9294.
- **September 10, 2013:** No changes
• January 1, 2014: Administrative Update: Replaced reimbursement code C9294 with J3060.
• September 9, 2014: No changes
• August 11, 2015: No changes
• January 1, 2016: Administrative change to rebranded template
• July 12, 2016: No changes
• July 11, 2017: No changes

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION
Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. They are used in conjunction with a Member’s benefit document and in coordination with the Member’s physician(s). The plan makes coverage decisions on a case-by-case basis considering the individual Member’s health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

This Pharmacy Medical Necessity Guideline does not apply to Uniformed Services Family Health Plan Members or to certain delegated service arrangements. Unless otherwise noted in the Member’s benefit document or applicable Pharmacy Medical Necessity Guideline, Pharmacy Medical Necessity Guidelines do not apply to CareLink℠ Members. For self-insured plans, drug coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a coverage guideline and a self-insured Member’s benefit document, the provisions of the benefit document will govern. Applicable state or federal mandates will take precedence.

For Tufts Health Plan Medicare Preferred, please refer to Tufts Health Plan Medicare Preferred Prior Authorization Criteria.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to Member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.