Cerezyme

Description

Cerezyme (imiglucerase)

Background
Gaucher disease is an inherited lysosomal storage disorder in humans that results in the inability to produce glucocerebrosidase, an enzyme necessary for fat metabolism. The enzyme deficiency causes lipids to collect in the spleen, liver, kidneys, and other organs. Accumulation of lipids in these areas results in the enlargement of the liver and spleen, anemia, thrombocytopenia, lung disease and bone abnormalities. Symptoms of Gaucher disease usually become apparent in early childhood or adolescence but can be diagnosed at any stage of life. It is important to begin intervention early to prevent damage to the liver and spleen (1).

Cerezyme is an injectable enzyme replacement product for the treatment of pediatric and adult patients with type 1 Gaucher disease. Cerezyme (imiglucerase for injection) catalyzes the hydrolysis of glucocerebrosidase to glucose and ceramide. In clinical trials, Cerezyme improved anemia and thrombocytopenia, reduced spleen and liver size, and decreased cachexia (1).

Regulatory Status
FDA-approved indication: Cerezyme is an analogue of the human enzyme β-glucocerebrosidase 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: (1)

1. Anemia
2. Thrombocytopenia
3. Bone disease
4. Hepatomegaly or splenomegaly
The most common adverse effects are infusion reactions and allergic reactions. Anaphylaxis has been observed in some patients (1).

In patients who developed IgG antibody to Cerezyme, an apparent effect on serum enzyme levels resulted in diminished volume of distribution and clearance and increased elimination half-life compared to patients without antibody (1).

The safety and effectiveness of Cerezyme has been established in patients between 2 and 16 years of age. Cerezyme has been administered to patients younger than 2 years of age, however the safety and effectiveness in patients younger than 2 has not been established (1).

Related policies
Cerdelga, Elelyso, VPRIV

Policy
This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Cerezyme may be considered medically necessary in patients with the diagnosis of type 1 Gaucher disease and if the conditions indicated below are met.

Cerezyme may be considered investigational for all other indications.

Prior-Approval Requirements

Diagnosis

Patient must have the following:

Type 1 Gaucher disease that results in one or more of the following:

1. Anemia
2. Thrombocytopenia
3. Bone disease
4. Hepatomegaly
5. Splenomegaly

AND the following:

NO dual therapy with another hydrolytic lysosomal glucocerebrosidase agent
Prior – Approval *Renewal* Requirements

Same as above

**Policy Guidelines**

**Pre - PA Allowance**

None

**Prior - Approval Limits**

Duration  2 years

**Prior – Approval *Renewal* Limits**

Same as above

**Rationale**

**Summary**

Gaucher disease is an inherited lysosomal storage disorder in humans that results in the inability to produce glucocerebrosidase, an enzyme necessary for fat metabolism. The enzyme deficiency causes lipids to collect in the spleen, liver, kidneys, and other organs. It is important to begin intervention early to prevent damage to the liver and spleen. In clinical trials, Cerezyme improved anemia and thrombocytopenia, reduced spleen and liver size, and decreased cachexia. Cerezyme is a form of the human lysosomal enzyme, glucocerebrosidase, and is effective in replacing the enzyme deficiency in type 1 (non-neuronopathic) Gaucher disease (1).

Prior approval is required to ensure the safe, clinically appropriate and cost effective use of Cerezyme while maintaining optimal therapeutic outcomes.

**References**


**Policy History**

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<tr>
<td>September 2011</td>
<td>New Policy</td>
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<tr>
<td>September 2012</td>
<td>Annual editorial review and reference update.</td>
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<tr>
<td>March 2013</td>
<td>Annual editorial review and reference update.</td>
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<td></td>
<td>Addition of conditions that result from Type 1 Gaucher Disease to criteria.</td>
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Section: Prescription Drugs  Effective Date: October 1, 2019
Subsection: Hematological Agents  Original Policy Date: September 8, 2011
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<tr>
<td>March 2014</td>
<td>Annual review</td>
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<td>December 2014</td>
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<td>December 2015</td>
<td>Annual editorial review and reference update&lt;br&gt;Addition of no dual therapy with another hydrolytic lysosomal glucocerebroside agent</td>
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<td>December 2016</td>
<td>Annual editorial review and reference update&lt;br&gt;Policy code changed from 5.10.04 to 5.85.04</td>
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<td>September 2019</td>
<td>Annual editorial review. Changed approval duration from lifetime to 2 years</td>
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**Keywords**

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on September 13, 2019 and is effective on October 1, 2019.