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5.85.004

Section:	Prescription Drugs	**Effective Date:**	July 1, 2023
Subsection:	Hematological Agents	**Original Policy Date:**	September 8, 2011
Subject:	Cerezyme	**Page:**	1 of 5

Last Review Date: June 15, 2023

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 catalyzes the hydrolysis of glucocerebroside 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 indications: Cerezyme is an analogue of the human enzyme β-glucocerebroside 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** 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:

1. **NO** dual therapy with another medication for Type 1 Gaucher disease (see Appendix 1)

Prior – Approval *Renewal* Requirements

Diagnosis

Patient must have the following:

Type 1 Gaucher disease

AND the following:

1. **NO** dual therapy with another medication for Type 1 Gaucher disease (see Appendix 1)

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

1. Cerezyme [package Insert]. Cambridge, MA: Genzyme Corporation; December 2022.

Policy History

Date	Action
September 2011	New Policy
September 2012	Annual editorial review and reference update.
March 2013	Annual editorial review and reference update. Addition of conditions that result from Type 1 Gaucher Disease to criteria.
March 2014	Annual review
December 2014	Annual editorial review and reference update
December 2015	Annual editorial review and reference update Addition of no dual therapy with another hydrolytic lysosomal glucocerebroside agent
December 2016	Annual editorial review and reference update Policy code changed from 5.10.04 to 5.85.04
September 2017	Annual editorial review and reference update
September 2018	Annual review and reference update
September 2019	Annual editorial review. Changed approval duration from lifetime to 2 years
September 2020	Annual review
March 2021	Annual review and reference update
March 2022	Annual review and reference update
March 2023	Annual review and reference update. Changed policy number to 5.85.004
June 2023	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 15, 2023 and is effective on July 1, 2023.

Appendix 1 - List of Medications for Type 1 Gaucher Disease

Generic Name	Brand Name
eliglustat	Cerdelga
imiglucerase	Cerezyme
miglustat	Zavesca
taliglucerase alfa	Elelyso
velaglucerase alfa	VPRIV