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5.85.014

Section:	Prescription Drugs	**Effective Date:**	April 1, 2024
Subsection:	Hematological Agents	**Original Policy Date:**	August 3, 2012
Subject:	Elelyso	**Page:**	1 of 5

Last Review Date: March 8, 2024

Elelyso

Description

Elelyso (taliglucerase alfa)

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).

Elelyso is an injectable enzyme replacement product for the treatment of adults with type 1 Gaucher disease. There are three clinical subtypes of Gaucher disease differentiated by the presence or absence of neurological involvement: type 1, type 2 and type 3. Type 1, known as non-neuronopathic, is the most common. There is insufficient evidence supporting the use of Elelyso for the treatment of type 2 and type 3 Gaucher disease (1).

Regulatory Status
FDA-approved indication: Elelyso is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy (ERT) for patients with a confirmed diagnosis of type 1 Gaucher disease (1).

The most common adverse effects are infusion reactions and allergic reactions. Anaphylaxis has been observed in some patients (1).

The safety of Elelyso has not been established in pediatric patients younger than 4 years of age (1).

Related policies
Cerdelga, Cerezyme, VPRIV, Zavesca

Policy

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

Elelyso may be considered **medically necessary** if the conditions indicated below are met.

Elelyso may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 4 years of age and older

Diagnosis

Patient must have the following:

Gaucher disease, Type 1

AND the following:

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

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. Elelyso is a form of the human lysosomal enzyme, glucocerebrosidase, and is effective in replacing the enzyme deficiency in type 1 (non-neuronopathic) Gaucher disease. The safety of Elelyso has not been established in pediatric patients younger than 4 years of age (1).

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

References

1. Elelyso [Package Insert]. New York, NY: Pfizer Labs; May 2023.

Policy History

Date	Action
July 2012	New Policy
March 2013	Annual editorial and reference update.
March 2014	Annual review
September 2014	Change of age limit to include pediatric patients
December 2014	Annual editorial 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 5.10.14 to 5.85.14
September 2017	Annual editorial review and reference update
September 2018	Annual editorial review and reference update
September 2019	Annual editorial review. Changed approval duration from lifetime to 2 years
September 2020	Annual review and reference update
March 2021	Annual review and reference update
March 2022	Annual review

March 2023	Annual review and reference update. Changed policy number to 5.85.014
June 2023	Annual review
March 2024	Annual review and reference update

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 8, 2024 and is effective on April 1, 2024.

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