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# 5.85.014

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<b>Section:</b>	Prescription Drugs	<b>Effective Date:</b>	April 1, 2025
<b>Subsection:</b>	Hematological Agents	<b>Original Policy Date:</b>	August 3, 2012
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**Last Review Date:** March 7, 2025

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## ElELYso

### Description

#### ElELYso (taliglucerase alfa)

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#### 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 the treatment of patients 4 years and older with a confirmed diagnosis of type 1 Gaucher disease (1).

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Elelyso has a boxed warning regarding hypersensitivity reactions including anaphylaxis. Elelyso should be administered in a healthcare setting with appropriate medical monitoring and support measures, including access to cardiopulmonary resuscitation equipment (1).

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

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### 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)

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### Prior-Approval *Renewal* Requirements

Same as above

### Policy Guidelines

#### Pre - PA Allowance

None

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## Prior - Approval Limits

Duration 2 years

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## 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; January 2025.

## 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 glucocerebrosidase 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

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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
June 2024	Annual editorial review and reference update
March 2025	Annual review

## Keywords

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**This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.**

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## Appendix 1 - List of Medications for Type 1 Gaucher Disease

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