

5.85.019

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<b>Section:</b>	Prescription Drugs	<b>Effective Date:</b>	July 1, 2024
<b>Subsection:</b>	Hematological Agents	<b>Original Policy Date:</b>	October 24, 2014
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**Last Review Date:** June 13, 2024

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## Zavesca

### Description

#### Zavesca (miglustat)

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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 fat materials (lipids) to collect and build up over time, causing problems in the spleen, liver, and bone marrow. Accumulation of lipids in these areas results in the enlargement of the liver and spleen, anemia, thrombocytopenia, lung disease and bone abnormalities (1).

Zavesca is an oral administration for the long-term treatment of adult patients with the type 1 form of Gaucher disease. The drug reduces the harmful buildup of the fatty materials by reducing the amount of glucosylceramide- based glycosphingolipids the body produces (1).

#### Regulatory Status

FDA-approved indication: Zavesca is a glucosylceramide synthase inhibitor indicated as monotherapy for treatment of adult patients with mild/moderate type 1 Gaucher disease for whom enzyme replacement therapy is not a therapeutic option (1).

People with type 1 Gaucher disease also may have lowered levels of hemoglobin (a substance in red blood cells) and platelets (blood-clotting cells) that may cause anemia (low red blood cell count) (1).

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Clinically significant adverse reactions may occur with Zavesca therapy including peripheral neuropathy, tremor, reduction in platelet count, diarrhea, and weight loss. Based on the severity of the adverse reaction, Zavesca therapy should have a dose reduction or discontinued. Patients with mild to moderate renal insufficiency should have a dose reduction. Use of Zavesca in patients with severe renal impairment (creatinine clearance < 30mL/min/1.73 m<sup>2</sup>) is not recommended. Therapy should be directed by physicians knowledgeable in the management of patients with Gaucher disease (1).

Safety and effectiveness of Zavesca in pediatric patients have not been established (1).

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#### Related policies

Cerdelga, Cerezyme, 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.*

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

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

### Prior-Approval Requirements

**Age** 18 years of age or older

#### Diagnosis

Patient must have the following:

Mild-to-moderate type 1 Gaucher disease

**AND ALL** the following:

1. Enzyme replacement therapy (such as Cerezyme, Elelyso, VPRIV) is not a therapeutic option (e.g., due to constraints such as allergy, hypersensitivity, or poor venous access)
2. **NO** dual therapy with another medication for Type 1 Gaucher disease (see Appendix 1)

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## Prior-Approval *Renewal* Requirements

**Age** 18 years of age or older

### 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

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### Prior-Approval *Renewal* Limits

Same as above

### Rationale

#### Summary

Zavesca is an oral administration for the long-term monotherapy treatment of adult patients with mild/moderate type 1 Gaucher disease for whom enzyme replacement therapy is not a therapeutic option due to constraints such as allergy, hypersensitivity, or poor venous access. Clinically significant adverse reactions may occur with Zavesca therapy including peripheral neuropathy, tremor, reduction in platelet count, diarrhea, and weight loss. Based on the severity of the adverse reaction, Zavesca therapy should have a dose reduction or discontinued. Patients with mild to moderate renal insufficiency should have a dose reduction and not recommended in patients with severe renal impairment. Therapy should be directed by physicians knowledgeable in the management of patients with Gaucher disease. Safety and effectiveness of Zavesca in pediatric patients have not been established (1).

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

## References

1. Zavesca [package Insert]. San Francisco, CA: Actelion Pharmaceuticals US Inc.; August 2022.

## Policy History

Date	Action
November 2014	Addition to PA
December 2014	Annual editorial review and reference update
December 2015	Annual review
December 2016	Annual review and reference update
	Policy number change from 5.10.19 to 5.85.19
September 2017	Annual editorial review and reference update Removal of Ceredase which is no long marketed
September 2018	Annual review and reference update
September 2019	Annual editorial review. Changed approval duration from lifetime to 2 years
September 2020	Annual review
December 2021	Annual review and reference update
December 2022	Annual review and reference update. Changed policy number to 5.85.019
June 2023	Annual review
June 2024	Annual review

## Keywords

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**This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 13, 2024 and is effective on July 1, 2024.**

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## 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