Zavesca

Description

Zavesca (miglustat)

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).
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 m²) 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).

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 as monotherapy in patients that are 18 years of age and older with the diagnosis of mild to moderate type 1 Gaucher disease and if the conditions indicated below are met.

Zavesca is considered investigational in patients less than 18 years of age and 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. **NOT** to be used concurrently with other medications for type 1 Gaucher diseases

**Prior–Approval **Renewal Requirements

**Age**  
18 years of age or older

**Diagnosis**

Patient must have the following:

- Type 1 Gaucher disease

**AND ALL** the following:

1. **NOT** to be used concurrently with other medications for type 1 Gaucher diseases

**Policy Guidelines**

**Pre - PA Allowance**

None

**Prior - Approval Limits**

**Duration**  
2 years

**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).

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

References

Policy History

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<tr>
<td>November 2014</td>
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<td>December 2015</td>
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<td></td>
<td>Policy number change from 5.10.19 to 5.85.19</td>
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<td>September 2017</td>
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<td></td>
<td>Removal of Ceredase which is no long marketed</td>
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<td>September 2018</td>
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<td>September 2019</td>
<td>Annual editorial review. Changed approval duration from</td>
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<td>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.