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 glucocerebrosidase-specific enzyme indicated for long-term enzyme replacement therapy (ERT) for patients with a confirmed diagnosis of type 1 Gaucher disease (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** in patients 4 years of age or older with a diagnosis of type 1 Gaucher disease and if the conditions indicated below are met.

Elelyso is considered **investigational** in patients under 4 years of age and 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:
  
  - NO dual therapy with another hydrolytic lysosomal glucocerebrosidase agent

**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

Policy History

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This policy was approved by the FEP® Pharmacy and Medical Policy Committee September 13, 2019 and is effective on October 1, 2019.