Lumizyme

Description

Lumizyme (alglucosidase alfa)

Background
Lumizyme is indicated for Pompe disease, a rare genetic disorder. In Pompe disease, a gene mutation prevents the body from making an enzyme, or making enough of the enzyme called acid alpha-glucosidase (GAA), necessary for proper muscle functioning. GAA is used by the heart and muscle cells to convert a form of sugar called glycogen into energy. Without the enzyme action, glycogen builds up in the cells and, ultimately, weakens the heart and muscles. Lumizyme replaces the deficient GAA, thereby reducing the accumulated glycogen in heart and skeletal muscle cells (1).

Regulatory Status
FDA approved indication: Lumizyme (alglucosidase alfa) is a hydrolytic lysosomal glycogen-specific enzyme indicated for patients with Pompe disease (acid α-glucosidase (GAA) deficiency) (1).

Acute cardiorespiratory failure has been observed in a few infantile-onset Pompe disease patients with underlying cardiac hypertrophy, possibly associated with fluid overload with intravenous administration of alglucosidase alfa (1).

Lumizyme has a boxed warning that anaphylactic, severe allergic and immune mediated reactions have been observed during administration and up to 3 hours after. Patients with acute underlying respiratory illness or compromised cardiac and/or respiratory function may be at risk
Patients should be monitored for IgG antibody formation every 3 months for 2 years and then annually thereafter. Testing for IgG titers may also be considered if patients develop allergic or other immune mediated reactions. Patients who experience anaphylactic or allergic reactions may also be tested for IgE antibodies to alglucosidase alfa and other mediators of anaphylaxis. Patients who develop IgE antibodies to alglucosidase alfa appear to be at a higher risk for the occurrence of anaphylaxis and severe allergic reactions. Therefore, these patients should be monitored more closely during administration of Lumizyme (1).

The safety and effectiveness of alglucosidase alfa have been established in pediatric patients with Pompe disease (1).

Related policies

Policy

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

Lumizyme may be considered medically necessary for patients with Pompe disease (acid α-glucosidase (GAA) deficiency) and if the conditions indicated below are met.

Lumizyme may be considered investigational in patients with all other indications.

Prior-Approval Requirements

Diagnosis

Patient must have the following:

Pompe disease (acid alpha-glucosidase (GAA) deficiency)

AND ALL of the following:
1. NO history of cardiac hypertrophy or cardiomyopathy
2. Monitored for IgG antibody formation every 3 months for 2 years and then annually thereafter
Prior – Approval *Renewal* Requirements

**Diagnosis**

Patient must have the following:

- Pompe disease (acid alpha-glucosidase (GAA) deficiency)

  **AND** the following:

- 1. Monitored for IgG antibody formation every year

**Policy Guidelines**

**Pre - PA Allowance**

None

**Prior - Approval Limits**

**Duration** 2 years

**Prior – Approval *Renewal* Limits**

Same as above

**Rationale**

**Summary**

Lumizyme (alglucosidase alfa) is a lysosomal glycogen-specific enzyme indicated for patients with Pompe disease (acid α-glucosidase (GAA) deficiency). Lumizyme has a boxed warning that anaphylactic, severe allergic and immune mediated reactions have been observed. Patients with acute underlying respiratory illness or compromised cardiac and/or respiratory function may be at risk of serious exacerbation of their cardiac or respiratory compromise during infusions. Appropriate medical support should be available during infusion. Patients should be monitored for IgG antibody formation every 3 months for 2 years and then annually thereafter (1).

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

**References**

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 6, 2019 and is effective on January 1, 2020.