BlueCross BlueShield Federal Employee Program

# LUMIZYME
# (alglucosidase alfa)

## RATIONALE FOR INCLUSION IN PA PROGRAM

### 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 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 of serious exacerbation of their cardiac or respiratory compromise during infusions. Appropriate medical support should be available during infusion (1).

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).

# LUMIZYME (alglucosidase alfa)

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

**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**

1. Lumizyme [package Insert]. Cambridge, MA: Genzyme Corporation; March 2024.