

## **Specialty Pharmacy Program**

### **Myozyme<sup>®</sup> and Lumizyme<sup>®</sup> (alglucosidase alfa)**

#### **DESCRIPTION**

Myozyme is a lysosomal glycogen-specific enzyme indicated for use in patients with Pompe disease (acid  $\alpha$ -glucosidase [GAA] deficiency). Myozyme has been shown to improve ventilator-free survival in patients with infantile-onset Pompe disease as compared to an untreated historical control, whereas use in patients with other forms of Pompe disease has not been adequately studied to assure safety and efficacy.

Lumizyme is indicated for patients 8 years and older with late-onset (non-infantile) Pompe disease who do not have evidence of cardiac hypertrophy. The safety and efficacy of Lumizyme have not been evaluated in controlled clinical trials in infantile onset patients, or in late-onset (non-infantile) patients less than 8 years of age.

Pompe disease (acid maltase deficiency, glycogen storage disease type II, GSD II, glycogenosis type II) is an inherited disorder of glycogen metabolism caused by the absence or marked deficiency of the lysosomal enzyme GAA. Myozyme and Lumizyme provide an exogenous source of GAA.

#### **APPROVAL DURATION**

Approval duration: lifetime

#### **APPROVAL CRITERIA**

Myozyme or Lumizyme may be approved based on the following criteria:

- I. Member has a diagnosis of Pompe disease
  - A. Diagnosis was confirmed by acid  $\alpha$ -glucosidase (GAA) enzyme activity testing or DNA testing
  - B. Prior to infusions, member will be pretreated with antihistamines and/or antipyretics