Prior Authorization Approval Criteria

*Lumizyme (alglucosidase alfa)*

**Generic Name:** alglucosidase alfa

**Brand Name:** Lumizyme

**Medication Class:** human enzyme (glycoprotein)

**FDA-approved uses:** treatment of patients with Pompe disease

**Available dosage forms:** 5mg/ml alglucosidase alfa solution

**Usual dose:** 20mg/kg infused intravenously every 2 weeks.

**Duration of Therapy:** Indefinite.

**Approximate cost:** $870.00/ 50mg vial. Cost for a 70kg patient would be $24,360 (based on AWP 2014) for 1 treatment or $48,720/month.

**Criteria for Use:** *(bullet points below are all inclusive unless otherwise noted)*

- The indicated diagnosis (including any applicable labs and/or tests) and medication usage must be supported by documentation from the patient’s medical records.
- Confirmed diagnosis of Pompe disease (α-glucosidase deficiency)
- Must be prescribed by a physician who specializes in Pompe disease.

**Criteria for continuation of therapy:**

- First renewal: Patient exhibits signs of improvement in 6 minute walk test (6MWT) and % predicted FVC.
- Subsequent renewals: Patient is tolerating and responding to medication and there continues to be a medical need for the medication.

**Caution:**

- Some patients have experienced life-threatening severe allergic (anaphylactic) reactions (hives, problems breathing, low blood pressure, throat and lip swelling), or severe skin reactions (e.g. deep skin tissue reaction with open sore) and systemic immune mediated reactions (e.g. kidney problems and skin rashes) during LUMIZYME infusions. Therefore, appropriate medical support measures should be readily available during your LUMIZYME infusion.
- Infantile-onset Pompe disease patients with compromised cardiac or respiratory function may be at risk of serious acute exacerbation of their cardiac or respiratory compromise due to fluid overload, and require additional monitoring.

**Contraindication:**

- There are currently no known contraindications to alglucosidase alfa therapy

**Not approved if:**

- Patient does not meet the above stated criteria.
Special considerations:

- Medical benefit.
- Myozyme is the other α-l-glucosidase alfa that is currently available but is reserved for treating infants and children, who have a more aggressive form of Pompe disease than that occurring in older children and adults. It is made in relatively small 160-liter batches.
- At study entry, the mean % predicted FVC in the sitting position among all patients was about 55%. After 78 weeks, the mean % predicted FVC increased to 56.2% for LUMIZYME-treated patients and decreased to 52.8% for placebo-treated patients indicating a LUMIZYME treatment effect of 3.4% (95% confidence interval: [1.3% to 5.5%]; p=0.004). Stabilization of % predicted FVC in the LUMIZYME-treated patients was observed.
- At study entry, the mean 6 minute walk test (6MWT) among all patients was about 330 meters. After 78 weeks, the mean 6MWT increased by 25 meters for LUMIZYME-treated patients and decreased by 3 meters for placebo-treated patients indicating a LUMIZYME treatment effect of 28 meters (95% confidence interval: [-1 to 52 meters]; p=0.06)

Authorization Approval Duration:

- 1 year

Fallon Health Pharmacy and Therapeutics Committee approval: ________________________________

Date: __________________________

Adopted: 12/14/11
Revised: 8/1/14