



## Prior Authorization Approval Criteria

### *Lumizyme (alglucosidase alfa)*

<b>Generic Name:</b>	alglucosidase alfa
<b>Brand Name:</b>	Lumizyme
<b>Medication Class:</b>	human enzyme (glycoprotein)
<b>FDA-approved uses:</b>	treatment of patients 8 years of age and older with late onset (non-infantile) Pompe disease who do not have evidence of cardiac hypertrophy.
<b>Available dosage forms:</b>	5mg/ml alglucosidase alfa solution
<b>Usual dose:</b>	20mg/kg infused intravenously every 2 weeks.
<b>Duration of Therapy:</b>	Indefinite.
<b>Approximate cost:</b> (based on AWP 2011)	\$840.00/ 50mg vial. Cost for a 70kg patient would be \$23,520.00 for 1 treatment or \$47,040.00/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 late onset of Pompe disease ( $\alpha$ -glucosidase deficiency) without evidence of cardiac hypertrophy.
- Must be 8 years of age or older.
- Must be prescribed by a physician who specializes in Pompe disease.
- Only prescribers and healthcare facilities who are enrolled in the LUMIZYME ACE Program may prescribe, dispense or administer LUMIZYME, and only patients who have been enrolled in the program by their doctors and meet all of the conditions of the program may receive LUMIZYME. For more information regarding the registry program visit [www.pomperegistry.com](http://www.pomperegistry.com) or by calling 1-800-745-4447.

**Criteria for continuation of therapy:**

- Patient exhibits signs of improvement in 6 minute walk test (6MWT) and % predicted FVC.

**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.

**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 alglucosidase 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. Lumizyme is made in 2000-liter batches in order to be made available for adults.
- Because there is a risk that the disease may progress rapidly in Pompe disease patients who are less than 8 years old, LUMIZYME is available only through a restricted distribution program called the LUMIZYME ACE Program. Only prescribers and healthcare facilities who are enrolled in this program may prescribe, dispense or administer LUMIZYME, and only patients who have been enrolled in the program by their doctors and meet all of the conditions of the program may receive LUMIZYME. Speak with your doctor for more information about how to enroll.
- 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)

FCHP Pharmacy and Therapeutics Committee approval: \_\_\_\_\_

Date: \_\_\_\_\_

Adopted: 12/14/11