



**Prior Authorization Approval Criteria**  
*Department of Pharmacy Services*

**Generic Name:** aglucosidase alfa

**Brand Name:** Myozyme

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

**FDA Approved Use:**

- To improve ventilator-free survival in patients with infantile-onset Pompe disease

**Available Dosage Forms:**

- 50mg, 20ml vials of lyophilized cake or powder for reconstitution for IV infusion with sterile water for injection, to be diluted with 0.9% NaCl for injection (Normal Saline) to a final concentration of 0.5 to 4mg/ml.

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

**Duration of Therapy:** Indefinite. Studies were performed on infants from 1 month to 3.5 years of age, and ranged from 52 to 106 weeks.

**Approximate Monthly Cost** (based on AWP 12/1/07):

\$2,100 per month based on a 7kg child

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

- Confirmed diagnosis of infant-onset of Pompe disease ( $\alpha$ -glucosidase deficiency)

**Not approved if:**

- Diagnosis is made after the age of 2 years of age (Risks and benefits of Myozyme treatment in juvenile-onset Pompe disease have not been established)

**Not approved for continued therapy if:**

- Patients who develop IgG antibodies to aglucosidase alfa at a sustained titer level of  $\geq$  12,800. Treated patients who experience decreasing motor activity should be tested for neutralization of enzyme uptake or activity. A majority of patients in the clinical trials developed antibodies, and most of those developed antibodies within the first 3 months of exposure.

**Approved to continue therapy if:**

- Patient remains free from requiring ventilator use
- Patient does not exhibit signs of decreasing motor activity

P&T Approval: \_\_\_\_\_ Date: \_\_\_\_\_