**LUMIZYME/MYOZYME (Alglucosidase alfa)**

**Effective Date:** 1/28/14
**Date Developed:** 1/28/14 by Catherine Sanders, MD
**Last Approval Date:** 1/26/16, 1/24/17, 1/23/18, 1/22/19

Alglucosidase alfa is a recombinant form of the enzyme acid alpha-glucosidase (GAA), which is required for glycogen cleavage. Due to an inherited GAA deficiency or absence, glycogen accumulates in the tissues of patients with Pompe disease, leading to progressive muscle weakness. In infantile-onset Pompe disease, glycogen accumulates in cardiac and skeletal muscles and hepatic tissue, leading to cardiomyopathy and respiratory failure. Juvenile- and adult-onset Pompe disease are limited to glycogen accumulation in skeletal muscle, leading to respiratory failure. Alglucosidase alfa binds to mannose-6-phosphate receptors on the cell surface, is internalized, and transported to lysosomes where it is activated for increased enzymatic glycogen cleavage.

**Pre-Authorization Criteria:**
Alglucosidase alfa is used for treatment of patients with Pompe disease in the following categories:
- Infantile-onset for children 1 month to 3.5 years of age (at first infusion)(Myozyme)
- Noninfantile, late-onset for patients 8 years of age and older without evidence of cardiac hypertrophy (Lumizyme)

VCHCP requires that Lumizyme/Myozyme be prescribed by a physician specializing in the condition being treated.

**Prescribing and Access Restrictions:**
As a requirement of the REMS program, access to this medication is restricted. Lumizyme® is available only through Lumizyme® ACE (Alglucosidase Alfa Control and Education) program; only trained and certified prescribers and healthcare facilities enrolled in the program may prescribe, dispense, or administer Lumizyme®. Patients must be enrolled in and meet all the conditions of the program to receive therapy. For enrollment, call 1-800-745-4447.
Access to Myozyme® is restricted by the manufacturer, and allowed only to patients <8 years of age with infantile-onset or late-onset Pompe disease (who are restricted from access to Lumizyme®) or to patients of any age with a diagnosis of infantile-onset Pompe disease or evidence of cardiac hypertrophy. To obtain Myozyme®, call 1-800-745-4447; no formal distribution program is established, but availability is controlled by Genzyme.

**Dosing: Adult:**
Replacement therapy for Pompe disease: I.V.:
- **Infantile-onset (Myozyme®; unlabeled use):** 20 mg/kg over ~4 hours every 2 weeks
- **Noninfantile, late-onset (Lumizyme®):** 20 mg/kg over ~4 hours every 2 weeks
Dosing: Pediatric:
Replacement therapy for Pompe disease: I.V.:
*Infantile-onset (Myozyme®)*:
Children 1 month to 3.5 years (at first infusion): 20 mg/kg over ~4 hours every 2 weeks
*Children >3.5 years (unlabeled use): 20 mg/kg over ~4 hours every 2 weeks*
*Noninfantile, late-onset (Lumizyme®)*:
Children <8 years: Not recommended
Children ≥ 8 years: Refer to adult dosing.

Dosing: Renal Impairment:
No dosage adjustment provided in the manufacturer’s labeling.

Dosing: Hepatic Impairment:
No dosage adjustment provided in the manufacturer’s labeling.

Dosage Forms: U.S.:
Excipient information presented when available (limited, particularly for generics); consult specific product labeling.
Solution Reconstituted, Intravenous [preservative free]:
Lumizyme: 50 mg (1 ea) [contains polysorbate 80]
Myozyme: 50 mg (1 ea)

Generic Equivalent Available: U.S.-No

Administration:
Infuse over ~4 hours; initiate at 1 mg/kg/hour. If tolerated, increase by 2 mg/kg/hour every 30 minutes to a maximum rate of 7 mg/kg/hour. Decrease rate or temporarily hold for infusion reactions. Infuse through a low protein-binding, 0.2 micron in-line filter. Monitor vital signs prior to each rate increase.
Compatibility
Stable in NS; do not infuse with other products.

Adverse Reactions:
>20%: tachycardia, bradycardia, flushing, fever, postprocedural pain, rash, diaper dermatitis, urticaria, diarrhea, vomiting, gastroenteritis, oral candidiasis, gastroesophageal reflux, constipation, anemia, catheter-related infections, myalgia, otitis media, hearing impairment, cough, pneumonia, upper respiratory tract infection, oxygen saturation decreased, pharyngitis, respiratory distress, respiratory failure, rhinorrhea, bronchiolitis, nasopharyngitis, tachypnea.
Other Less Common Serious Reactions: Infusion reaction-severe, anaphylaxis, hypersensitivity reaction, immune-mediated reaction, nephrotic syndrome.

U.S.BOXED WARNING:
Restricted distribution program (ACE) to minimize rapid disease progression risk in patients < 8 years old for whom safety/efficacy no established; prescribers, healthcare facilities must enroll at 1-800-745-4447; administered only to program-qualified/enrolled patients by healthcare providers enrolled in the program.
Life threatening anaphylactic reactions, severe hypersensitivity reactions, immune-mediated reactions have occurred during infusion; administer where appropriate medical support available. Use with caution in patients with compromised cardiac function and/or compromised respiratory function; risk of acute cardiorespiratory failure secondary to infusion-related reactions may be increased.

References:
5. [www.uptodate.com](http://www.uptodate.com): Alglucosidase alfa: Drug Information
6. [www.epocrates.com](http://www.epocrates.com): Lumizyme Drug information

REVISION HISTORY:
Date Reviewed/No Updates: 1/13/15 by C. Sanders, MD
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