

Policy: MBP 83.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Lumizyme (alglucosidase alfa)

I. Policy:

Lumizyme (alglucosidase alfa)

II. Purpose/Objective:

To provide a policy of coverage regarding Lumizyme (alglucosidase alfa)

III. Responsibility:

- A. Medical Directors
- B. Medical Management
- C. Pharmacy Department

IV. Required Definitions

1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than
3. the department requiring/authoring the policy.
4. Devised – the date the policy was implemented.
5. Revised – the date of every revision to the policy, including typographical and grammatical changes.
6. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions

Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:

- a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
- b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
- c. in accordance with current standards good medical treatment practiced by the general medical community;
- d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
- e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment

Medical Necessity shall mean a service or benefit that is compensable under the Medical Assistance Program and if it meets any one of the following standards:

- (i) the service or benefit will, or is reasonably expected to, prevent the onset of an illness, condition or disability.
- (ii) the service or benefit will, or is reasonably expected to, reduce or ameliorate the physical, mental or development effects of an illness, condition, injury or disability.
- (iii) the service or benefit will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for members of the same age

DESCRIPTION:

Lumizyme (alglucosidase alfa) is a lysosomal glycogen-specific enzyme indicated for patients with Pompe disease [acid alpha-glucosidase (GAA) deficiency]. Lumizyme (alglucosidase alfa) provides an exogenous source of GAA allowing conversion of glycogen to energy in heart and muscle cells.

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

Lumizyme (alglucosidase alfa) will be considered medically necessary when all of the following criteria are met:

- Physician provided documentation of a diagnosis of late-onset (non-infantile) Pompe disease **OR** a diagnosis of infantile-onset Pompe disease supported by:
 - GAA assay performed on dried blood spots, skin fibroblasts or muscle biopsy; **and**
 - Baseline pulmonary function testing (PFT) and muscle strength evaluation; **and**
 - For late-onset Pompe disease only – Genetic testing to identify the specific mutation to confirm the diagnosis of late-onset Pompe disease; **and**
- Physician provided documentation of a consultation with a metabolic specialist and/or biochemical geneticist; **and**
- Dosing calculation is based on the “Devine formula”^{1,2} which is defined as:
Men: Ideal Body Weight (in kilograms) = 50 + 2.3 kg per inch over 5 feet.
Women: Ideal Body Weight (in kilograms) = 45.5 + 2.3 kg per inch over 5 feet.

¹ **Note:** Use of the Devine formula for dosing calculation is supported the Duke University Medical Center’s Pompe Disease Clinic

² Genzyme acknowledges that some physicians dose Lumizyme based on ideal body weight although the trial that led to approval of Lumizyme used actual body weight. Additionally, Genzyme reports that at the 2012 American College of Medical Genetics conference, an abstract was presented in which the authors showed their research of dosing Lumizyme in a small population of overweight/obese patients with late-onset Pompe disease.

AUTHORIZATION DURATION: Initial approval will be for 6 months or less if the reviewing provider feels it is medically appropriate. Subsequent approvals will be for an additional 6 months or less if the reviewing provider feels it is medically appropriate and will require medical record documentation of continued disease improvement or lack of disease progression as evidenced by results of appropriate monitoring studies, including

- Upright and supine PFT’s done at a minimum of 6 month intervals
- Muscle strength evaluations
- Echocardiograms

The medication will no longer be covered if patient experiences toxicity or worsening of disease.

LINE OF BUSINESS:

Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

This policy will be revised as necessary and reviewed no less than annually.

Devised: 9/8/10

Revised: 08/14, 09/26/14, 5/16 (added testing)

Reviewed: 5/12, 08/14, 09/26/14, 1/20/15, 3/16, 3/30/17, 3/29/18, 3/28/19, 1/1/20, 1/1/21, 12/21/21