Clinical Policy: Alglucosidase alfa (Lumizyme, Myozyme)
Reference Number: ERX.SPMN.144
Effective Date: 10/16
Last Review Date: 09/16

See Important Reminder at the end of this policy for important regulatory and legal information.

Policy/Criteria
It is the policy of health plans affiliated with Envolve Pharmacy Solutions® that alglucosidase alfa (Lumizyme®, Myozyme®) is medically necessary when the following criteria are met:

I. Initial Approval Criteria
   A. Pompe Disease (GAA Deficiency) (must meet all):
      1. Age ≥ 1 month;
      2. Diagnosis of Pompe disease confirmed by one of the following:
         a. Enzyme assay confirming low acid-α-glucosidase (GAA) activity and GAA activity testing in fibroblast;
         b. DNA testing;
      3. If Myozyme is prescribed, patient has infantile-onset Pompe disease.

      Approval duration: 6 months

   B. Other diagnoses/indications: Refer to ERX.SPMN.16 - Global Biopharm Policy.

II. Continued Approval
   A. Pompe Disease (GAA Deficiency) (must meet all):
      1. Currently receiving medication via health plan benefit or member has previously met all initial approval criteria.

      Approval duration: 12 months

   B. Other diagnoses/indications (must meet 1 or 2):
      1. Currently receiving medication via health plan benefit and documentation supports positive response to therapy; or
      2. Refer to ERX.SPMN.16 - Global Biopharm Policy.
Background

**Description/Mechanism of Action:**
Pompe disease, also known as acid maltase deficiency or glycogen storage disease type II, is an autosomal recessive muscle disorder caused by a deficiency of the lysosomal enzyme acid-α-glucosidase (GAA). This enzymatic deficiency results in lysosomal glycogen accumulation in predominantly skeletal, cardiac and smooth muscle. Subtypes, based on degree of organ involvement and age of onset, are broadly classified as infantile onset (a relatively more severe course in infancy with prominent cardiorespiratory complications) and late onset (a less severe course presenting from childhood through the adult years and marked primarily by skeletal muscle involvement). Diagnosis is confirmed by an absence of or reduced GAA activity in fibroblasts. Mutational analysis can help inform diagnosis, especially in cases where some uncertainty remains around GAA enzyme activity findings. Improvements have been observed in survival and cardiac and skeletal muscle function with the advent of enzyme replacement therapy.\(^1\) Lumizyme and Myozyme, both recombinant forms of GAA, are indicated as enzymatic replacement therapy in Pompe disease – Lumizyme for both infantile and late-onset forms; Myozyme for infantile onset only.

Alglucosidase alfa provides an exogenous source of GAA. Binding to mannose-6-phosphate receptors on the cell surface has been shown to occur via carbohydrate groups on the GAA molecule, after which it is internalized and transported into lysosomes, where it undergoes proteolytic cleavage that results in increased enzymatic activity. It then exerts enzymatic activity in cleaving glycogen.

**FDA Approved Indication(s):**
Lumizyme and Myozyme, are enzymes/intravenous solutions indicated for:
- Pompe disease (GAA deficiency) [Myozyme is indicated for infantile-onset only].

Appendices

**Appendix A: Abbreviation Key**
GAA: acid-α-glucosidase

**Coding Implications**
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.
### HCPCS Codes

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<tr>
<th>HCPCS Codes</th>
<th>Description</th>
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<tr>
<td>J0220</td>
<td>Injection, alglucosidase alfa, 10 mg, not otherwise specified</td>
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<tr>
<td>J0221</td>
<td>Injection, alglucosidase alfa, (Lumizyme), 10 mg</td>
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### Reviews, Revisions, and Approvals

<table>
<thead>
<tr>
<th>Policy split from USS.SPMN.33 Lysosomal Storage Disorders and converted to new template. Added age restriction per PI. Modified diagnosis criteria to require only 1 test rather than 2. Modified approval duration to 6 months for initial and 12 months for re-auth.</th>
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### References


### Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.

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