

# SmartPA Criteria Proposal

<b>Drug/Drug Class:</b>	Pompe Disease Clinical Edit
<b>First Implementation Date:</b>	April 21, 2022
<b>Proposed Date:</b>	December 15, 2022
<b>Prepared for:</b>	MO HealthNet
<b>Prepared by:</b>	MO HealthNet/Conduent
<b>Criteria Status:</b>	<input checked="" type="checkbox"/> Existing Criteria <input type="checkbox"/> Revision of Existing Criteria <input type="checkbox"/> New Criteria

## Executive Summary

**Purpose:** Ensure appropriate utilization and control of agents used for Pompe Disease.

**Why Issue Selected:** Pompe disease is a rare, genetic, lysosomal storage disease caused by a deficiency of the enzyme acid alpha-glucosidase (GAA), which is responsible for converting glycogen into glucose within the lysosomes. Deficiency of GAA results in lysosomal accumulation of glycogen in the skeletal, cardiac, and smooth muscles. Incidence is estimated at approximately 1 in 40,000 individuals in the United States. Pompe disease is classified as either infantile-onset (IOPD) or late-onset (LOPD). IOPD is characterized as complete or near-complete deficiency of GAA, while LOPD is characterized by partial deficiency of GAA. LOPD applies to all cases in which hypertrophic cardiomyopathy (HCM) did not manifest or was not diagnosed at or under 1 year of age, as well as all cases with symptom onset after 1 year of age. The American Association of Neuromuscular and Electrodiagnostic Medicine (AANEM) recommends Enzyme Replacement Therapy (ERT) at symptom onset and/or at onset of detectable proximal muscle weakness or reduced forced vital capacity (FVC) in either upright or supine position. Generally, after 1 year of ERT treatment patients should be reevaluated to determine whether ERT should be continued. There is currently no evidence to give preference to one ERT over another in the treatment of late-onset Pompe disease.

Approved by the FDA in May 2010, Lumizyme® (alglucosidase alfa) is indicated for patients with both infantile-onset and late-onset Pompe disease. Lumizyme is an exogenous source of GAA resulting in increased GAA enzymatic activity and glycogen cleavage. Treatment with Lumizyme is associated with prolonging life by improving cardiac, respiratory, and skeletal muscle function.

FDA approved in August 2021, Nexvzyme™ (avalglucosidase alfa-ngpt) is indicated for the treatment of patients 1 year of age and older with LOPD. Nexvzyme is an exogenous source of GAA and binds to M6P receptors on the cell surface, it is then internalized and transported into lysosomes where it undergoes proteolytic cleavage that results in increased GAA enzymatic activity and glycogen cleavage.

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Due to the high cost and specific approved indications, MO HealthNet will impose clinical criteria to ensure appropriate utilization of agents for Pompe disease.

Program-Specific Information:

Date Range FFS 10/1/2021 to 9/30/2022			
Drug	Claims	Spend	Average of PAID
LUMIZYME 50 MG/VIAL	155	\$5,698,343.88	\$36,763.51
NEXVIAZYME 100 MG/VIAL	38	\$479,497.03	\$12,618.34

Type of Criteria: ☐ Increased risk of ADE ☐ Preferred Drug List  
☒ Appropriate Indications ☒ Clinical Edit

Data Sources: ☐ Only Administrative Databases

## Setting & Population

- Drug class for review: agents for Pompe disease
- Age range: All appropriate MO HealthNet participants

## Approval Criteria

### Initial Therapy:

- Prescribed by or in consultation with a neurologist, geneticist, or other specialist in the treated disease state **AND**
- Documented diagnosis of Pompe disease **AND**
- For Nexviazyme
  - Documented medical reason why participant cannot use Lumizyme **AND**
  - Participant aged  $\geq 1$  year **AND**
  - Diagnosis of late-onset Pompe Disease, as evidenced by the following:
    - Enzyme assay showing a deficiency of acid-alpha glucosidase (GAA) activity in the blood, skin, or muscle **OR**
    - Genetic testing showing a pathogenic variant of the GAA gene

### Continuation of Therapy:

- Initial approval is for 1 year, renewal of prior authorization may be authorized if participant is compliant on current therapy regimen and documentation of benefit of therapy is received

## Denial Criteria

- Therapy will be denied if all approval criteria are not met
- For Lumizyme: concomitant use of Nexviazyme
- For Nexviazyme: concomitant use of Lumizyme

## Required Documentation

Laboratory Results: ☐  
 MedWatch Form: ☐

Progress Notes: ☐  
 Other: ☒

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## Disposition of Edit

Denial: Exception code "0682" (Clinical Edit)  
Rule Type: CE

## Default Approval Period

1 year

## References

- Nexvazyme [package insert]. Cambridge, MA: Sanofi Genzyme Corporation; August 2021.
- Lumizyme [package insert]. Cambridge, MA: Sanofi Genzyme Corporation; May 2022.
- IPD Analytics. Endocrinology and Metabolic Agents: Pompe Disease. August 2021.
- IPD Analytics. New Drug Review: Nexvazyme (avalglucosidase alfa-ngpt). August 2021.
- "FDA approves Nexvazyme (avalglucosidase alfa-ngpt), an important new treatment option for late-onset Pompe disease." Sanofi Press Release. Available online: <https://www.globenewswire.com/news-release/2021/08/06/2276588/0/en/FDA-approves-Nexvazyme-avalglucosidase-alfa-ngpt-an-important-new-treatment-option-for-late-onset-Pompe-disease.html>. Accessed November 2022.
- "FDA approved Sanofi Genzyme's Nexvazyme for treatment of Pompe disease." Muscular Dystrophy Association. Available online: <https://strongly.mda.org/fda-approves-sanofi-genzymes-nexvazyme-for-treatment-of-pompe-disease/>. Accessed November 2022.
- Culper E, Berger K, Leshner R, et al. Consensus Treatment Recommendations for Late-Onset Pompe Disease. *Muscle Nerve* 45:319-333, 2012.
- Pompe Disease. National Organization for Rare Disorders. Available online: <https://rarediseases.org/rare-diseases/pompe-disease/>. Accessed: November 2022.