HIGHMARK WHOLECARE

Updated: 07/2025
PARP Approved: 09/2025

Prior Authorization Criteria

Enzyme Replacement Therapy, Pompe Disease

All requests for Enzyme Replacement Therapy for Pompe Disease require a prior authorization and will be screened for medical necessity and appropriateness using the criteria listed below.

Enzyme Replacement Therapy for Pompe Disease medications include Lumizyme (alglucosidase alfa) and Nexviazyme (avalglucosidase alfa-ngpt). New products with this classification will require the same documentation.

Enzyme Replacement Therapy for Pompe Disease Prior Authorization Criteria:

Coverage may be provided with a diagnosis of **Pompe Disease** and the following criteria is met:

- Documentation of diagnosis of Pompe Disease (GAA deficiency) confirmed by ONE of the following:
    - Deficiency of acid alpha-glucosidase enzyme activity OR
    - Detection of pathogenic variants in the GAA gene by molecular genetic testing
- Must be age-appropriate according to FDA-approved labeling, nationally recognized compendia, or evidence-based practice guidelines
- Medication must be prescribed by or in consultation with a metabolic specialist and/or biochemical geneticist.
- Documentation of baseline values for one or more of the following:
    - Infantile-onset disease: muscle weakness, motor function, respiratory function, cardiac involvement, percent predicted forced vital capacity (FVC) OR
    - Late-onset (non-infantile) disease: percent predicted forced vital capacity (FVC), walking distance or 6-minute walk test (6MWT) or gastrointestinal symptoms
        - note: 6MWT excluded for members at an age not able to walk
- **Initial Duration of Approval:** 12 months
- **Reauthorization criteria**
    - Documentation the member demonstrates a clinical benefit to therapy compared to pre-treatment baseline in one or more of the following:
        - Infantile-onset disease: stabilization or improvement in muscle weakness, motor function, respiratory function, cardiac involvement, or FVC OR
        - Late-onset (non-infantile) disease: stabilization or improvement in FVC and/or 6MWT and signs/symptoms of the condition
- **Reauthorization Duration of approval:** 12 months

Coverage may be provided for any non-FDA labeled indication if it is determined that the use is a medically accepted indication supported by nationally recognized pharmacy compendia or peer-reviewed medical literature for treatment of the diagnosis(es) for which it is prescribed. These requests will be reviewed on a case by case basis to determine medical necessity.

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When criteria are not met, the request will be forwarded to a Medical Director for review. The physician reviewer must override criteria when, in their professional judgment, the requested medication is medically necessary.

ENZYME REPLACEMENT THERAPY, POMPE DISEASE
PRIOR AUTHORIZATION FORM

Please complete and fax all requested information below including any progress notes, laboratory test results, or chart documentation as applicable to Highmark Wholecare Pharmacy Services. **FAX:** (888) 245-2049
If needed, you may call to speak to a Pharmacy Services Representative. **PHONE**: (800) 392-1147 Mon – Fri 8:30am to 5:00pm

PROVIDER INFORMATION

Requesting Provider:	Provider NPI:
Provider Specialty:	Office Contact:
State license #:	Office NPI:
Office Address:	Office Phone:
	Office Fax:

MEMBER INFORMATION

Member Name:	DOB:	
Member ID:	Member weight:	Height:

REQUESTED DRUG INFORMATION

Medication:	Strength:	
Directions:	Quantity:	Refills:
Is the member currently receiving requested medication? ☐ Yes ☐ No	Date Medication Initiated:	

Billing Information

This medication will be billed: ☐ at a pharmacy **OR** ☐ medically, JCODE: _____

Place of Service: ☐ Hospital ☐ Provider's office ☐ Member's home ☐ Other

Place of Service Information

Name:	NPI:
Address:	Phone:

MEDICAL HISTORY (Complete for ALL requests)

Diagnosis: ☐ Infantile-onset Pompe disease ☐ Late-onset (non-infantile) Pompe disease ICD-10: _____

Does the member have confirmed testing of deficiency of acid alpha-glucosidase enzyme activity OR detection of pathogenic variants in the GAA gene by molecular genetic testing? ☐ Yes ☐ No

For infantile-onset disease: does the member have documented baseline values for one or more of the following: muscle weakness, motor function, respiratory function, cardiac involvement, percent predicted forced vital capacity (FVC)?
☐ Yes ☐ No

For late-onset (non-infantile) disease: does the member have documented baseline values for one or more of the following: percent predicted forced vital capacity (FVC), walking distance or 6-minute walk test (6MWT) or gastrointestinal symptoms?
☐ Yes ☐ No

CURRENT or PREVIOUS THERAPY

Medication Name	Strength/ Frequency	Dates of Therapy	Status (Discontinued & Why/Current)

REAUTHORIZATION

Please indicate which of the following apply as a result of treatment:

☐ For infantile-onset disease: Is there an improvement from the members baseline values for one or more of the following: muscle weakness, motor function, respiratory function, cardiac involvement, percent predicted forced vital capacity (FVC)?
☐ Yes ☐ No

☐ For late-onset (non-infantile) disease: Is there an improvement from the members baseline values for one or more of the following: percent predicted forced vital capacity (FVC), walking distance or 6-minute walk test (6MWT) or gastrointestinal symptoms? ☐ Yes ☐ No

SUPPORTING INFORMATION or CLINICAL RATIONALE	
Prescribing Provider Signature	Date