



Updated: 07/2025
DMMA Approved: 07/2025

**Request for Prior Authorization for Enzyme Replacement Therapy, Pompe Disease
Website Form – www.highmarkhealthoptions.com
Submit request via: Fax - 1-855-476-4158**

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

*Enzyme Replacement Therapy, 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, 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



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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.

Drugs are authorized in generic form unless the branded product is on the preferred drug list or the prescriber has indicated in writing that the branded product is medically necessary. If only the branded product is on the preferred drug list, the generic form will be considered non-preferred and shall not require the prescriber to indicate in writing that the branded product is medically necessary.



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**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 Health Options Pharmacy Services. **FAX:** (855) 476-4158
If needed, you may call to speak to a Pharmacy Services Representative.
PHONE: (844) 325-6251 Monday through Friday 8:00am to 7:00pm

PROVIDER INFORMATION

| | |
|----------------------|-----------------|
| Requesting Provider: | NPI: |
| Provider Specialty: | Office Contact: |
| 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? <input type="checkbox"/> Yes <input type="checkbox"/> No Date Medication Initiated: | | |
| Is this medication being used for a chronic or long-term condition for which the medication may be necessary for the life of the patient? <input type="checkbox"/> Yes <input type="checkbox"/> No | | |

Billing Information

| |
|--|
| This medication will be billed: <input type="checkbox"/> at a pharmacy OR <input type="checkbox"/> medically, JCODE: |
| Place of Service: <input type="checkbox"/> Hospital <input type="checkbox"/> Provider's office <input type="checkbox"/> Member's home <input type="checkbox"/> 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

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 |
| | |



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