ATRIO HEALTH PLANS

Pompe Disease

Nexviazyme (Avalglucosidase Alfa-ngpt) J0219, Lumizyme (alglucosidase alfa) J0220, Opfolda (miglustat) J1202, Pombiliti (cipaglucosidase alfa-atga) J1203

Prior Authorization Request

Medicare Part B Form

*Instructions: * Indicates required information – Form may be returned if required information is not provided. Please fax this request to the appropriate fax number listed at the bottom of the page.*

☐	**Standard Request– (72 Hours)**	☐	**Urgent Request** (standard time frame could place the member's life, health or ability in serious jeopardy)
	Date Requested _____		
	Requestor _____ Clinic name: _____ Phone / Fax		

MEMBER INFORMATION

*Name:_____ *ID#:_____ *DOB:_____

PRESCRIBER INFORMATION

*Name:_____ ☐MD ☐FNP ☐DO ☐NP ☐PA *Phone:_____

*Address:_____ *Fax:_____

DISPENSING PROVIDER / ADMINISTRATION INFORMATION

*Name:_____ Phone:_____

*Address:_____ Fax:_____

PROCEDURE / PRODUCT INFORMATION

HCPC Code	Name of Drug	Dose (Wt: _____ kg Ht:_____)	Frequency	End Date if known

☐ **Self-administered** ☐ **Provider-administered** ☐ **Home Infusion**

☐Chart notes attached. **Other important information:**_____

Diagnosis: ICD10: _____ **Description**: _____

☐ Provider attests the diagnosis provided is an FDA-Approved indication for this drug

CLINICAL INFORMATION

☐ **New Start or Initial Request: (Clinical documentation required for all requests)**

☐ **Nexviazyme**

- ☐ Individual has a diagnosis of non-infantile onset (late-onset) Pompe disease as confirmed by all the following:
  - ☐ Documentation is provided that individual has a GAA enzyme assay which shows reduced enzyme activity less than 40% of the lab specific normal mean value; AND
  - ☐ Documentation is provided that individual has a second GAA enzyme activity assay in a separate sample (from purified lymphocytes, fibroblasts or muscle) or by GAA sequencing (AANEM 2009); AND
  - ☐ Forced vital capacity (FVC) 30 – 85% of predicted value, and documentation is provided; AND
  - ☐ Ability to walk 40 meters on a 6- minute walk test (w/o assistive devices), with documentation

☐ **Lumizyme**

☐ Individual has a diagnosis of infantile-onset Pompe disease as confirmed by all of the following:

- ☐ Documentation is provided that individual has acid alpha-glucosidase deficiency (GAA) activity in skin fibroblasts of less than 1% of the normal mean or by GAA gene sequencing; AND
- ☐ Individual has symptoms (for example respiratory and/or skeletal muscle weakness); AND
- ☐ Confirmed evidence of hypertrophic cardiomyopathy;

☐ Individual has a diagnosis of non-infantile onset (late-onset) Pompe disease as confirmed by::

- ☐ A. Documentation is provided that individual has a GAA enzyme assay which shows reduced enzyme activity less than 40% of the lab specific normal mean value; AND
- ☐ B. Documentation is provided that individual has a second GAA enzyme activity assay in a separate sample (from purified lymphocytes, fibroblasts or muscle) or by GAA sequencing; AND
- ☐ C. Forced vital capacity (FVC) 30 – 79% of predicted value, and documentation is provided; AND
- ☐ D. Ability to walk 40 meters on a 6- minute walk test (assisted devices permitted), and documentation is provided; AND
- ☐ E. Muscle weakness in the lower extremities.

☐ **Opfolda + Pombiliti**

☐ Individual has a diagnosis of infantile-onset Pompe disease as confirmed by all of the following:

- ☐ Diagnosis of late-onset Pompe disease **AND**
- ☐ Patient will use Opfolda (miglustat) in combination with Pombiliti (cipaglucosidase alfa-atga)

☐ Continuation Requests: (Clinical documentation required for all requests)

☐ **Nexviazyme**

☐ Individuals with non-infantile onset (late-onset) Pompe disease are responding to therapy (including improvement, stabilization, or slowing of disease progression).

☐ **Lumizyme**

- ☐ Individuals are using Lumizyme for the treatment of infantile-onset Pompe disease; OR
- ☐ Individuals with non-infantile onset (late-onset) Pompe disease are responding to therapy (including improvement, stabilization, or slowing of disease progression).

☐ **Opfolda + Pombiliti**

- ☐ Documentation of positive clinical response to Opfolda plus Pombiliti AND
- ☐ Opfolda continues to be prescribed in combination with Pombiliti

ACKNOWLEDGEMENT

Request By (Signature Required): _____Date:_____ /____ /____

Any person who knowingly files a request for authorization of coverage of a medical procedure or service with the intent to injure, defraud or deceive any insurance company by providing materially false information or conceals material information for the purpose of misleading, commits a fraudulent insurance act, which is a crime and subjects such person to criminal and civil penalties. **THIS AUTHORIZATION IS NOT A GUARANTEE OF PAYMENT.** PAYMENT IS BASED ON BENEFITS IN EFFECT AT THE TIME OF SERVICE, MEMBER ELIGIBILITY AND MEDICAL NECESSITY.

Prior Authorization Group – Pompe Disease Drug PA

Drug Name(s):

NEXVIAZYME	**AVALGLUCOSIDASE ALFA-NGPT**
LUMIZYME	**ALGLUCOSIDASE ALFA**
OPFOLDA	**MIGLUSTAT**
POMBILITI	**CIPAGLUCOSIDASE ALFA-ATGA**

Criteria for approval of Non-Formulary/Preferred Drug:

1. Prescribed for an approved FDA diagnosis (as listed below):
2. Member does not have any clinically relevant contraindications, or CMS/Plan exclusions, to the requested drug.

- If the member meets all these criteria, they may be approved by the Plan for the requested drug.
- Quantity limits and Tiering will be determined by the Plan.
- Continuation Requests: Provider must verify continued clinical benefit in confirmatory trial(s).

Exclusion Criteria:

N/A

Prescriber Restrictions:

Clinical Geneticist or another related specialist

Coverage Duration:

Approval will be for 12 months

FDA Indications:

Nexviazyme

- Pompe disease, late-onset

Lumizyme

- Pompe disease

Opfolda + Pombiliti

Pompe disease, late-onset, In combination with migLUstat; in patients weighing 40 kg or greater and who are not improving with current enzyme replacement therapy

Off-Label Uses:

N/A

Age Restrictions:

N/A

Other Clinical Consideration:

Nexviazyme

- Black Box Warning:
  - Warning: Severe hypersensitivity reactions, infusion-associated reactions, and risk of acute cardiorespiratory failure in susceptible patients
  - Hypersensitivity Reactions including Anaphylaxis
  - Patients treated with avalglucosidase alfa-ngpt have experienced life-threatening hypersensitivity reactions, including anaphylaxis. Appropriate medical monitoring and support measures, including cardiopulmonary resuscitation equipment, should be readily available during avalglucosidase alfa-ngpt administration. If a

severe hypersensitivity reaction (e.g., anaphylaxis) occurs, discontinue avalglucosidase alfa-ngpt immediately and initiate appropriate medical treatment. In patients with severe hypersensitivity reaction, a desensitization procedure to avalglucosidase alfa-ngpt may be considered.
  - Infusion-Associated Reactions (IARs)
  - Patients treated with avalglucosidase alfa-ngpt have experienced severe IARs. If severe IARs occur, consider immediate discontinuation of avalglucosidase alfa-ngpt, initiation of appropriate medical treatment, and the benefits and risks of readministering avalglucosidase alfa-ngpt following severe IARs. Patients with an acute underlying illness at the time of avalglucosidase alfa-ngpt infusion may be at greater risk for IARs. Patients with advanced Pompe disease may have compromised cardiac and respiratory function, which may predispose them to a higher risk of severe complications from IARs.
  - Risk of Acute Cardiorespiratory Failure in Susceptible Patients
  - Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function for whom fluid restriction is indicated may be at risk of serious exacerbation of their cardiac or respiratory status during avalglucosidase alfa-ngpt infusion. More frequent monitoring of vitals should be performed during avalglucosidase alfa-ngpt infusion in such patients

Nexviazyme

- Black Box Warning:
  - Life-threatening anaphylactic reactions and severe hypersensitivity reactions, presenting as respiratory distress, hypoxia, apnea, dyspnea, bradycardia, tachycardia, bronchospasm, throat tightness, hypotension, angioedema (including tongue or lip swelling, periorbital edema, and face edema), and urticaria, have occurred in some patients during and after alglucosidase alfa infusions. Immune-mediated reactions presenting as proteinuria, nephrotic syndrome, and necrotizing skin lesions have occurred in some patients following alglucosidase alfa treatment. Closely observe patients during and after alglucosidase alfa administration and be prepared to manage anaphylaxis and hypersensitivity reactions. Inform patients of the signs and symptoms of anaphylaxis, hypersensitivity reactions, and immune-mediated reactions and have them seek immediate medical care should signs and symptoms occur. Infantile-onset Pompe disease patients with compromised cardiac or respiratory function may be at risk of serious acute exacerbation of their cardiac or respiratory compromise due to fluid overload, and require additional monitoring

Nexviazyme

- Black Box Warning:
  - Severe Hypersensitivity Reactions, Infusion-Associated Reactions, and Risk of Acute Cardiorespiratory Failure in Susceptible Patients
  - Hypersensitivity Reactions Including Anaphylaxis
  - Patients treated with cipaglucosidase alfa-atga have experienced life-threatening hypersensitivity reactions, including anaphylaxis. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during cipaglucosidase alfa-atga administration. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, cipaglucosidase alfa-atga should be discontinued immediately, and appropriate medical treatment should be initiated. In patients with severe hypersensitivity reaction, desensitization measures to cipaglucosidase alfa-atga may be considered.
  - Infusion-Associated Reactions (IARs)
  - Patients treated with cipaglucosidase alfa-atga have experienced severe IARs. If severe IARs occur, immediately discontinue the cipaglucosidase alfa-atga infusion, initiate appropriate medical treatment, and assess the benefits and risks of readministering cipaglucosidase alfa-atga following severe IARs. Patients with an acute underlying illness at the time of cipaglucosidase alfa-atga infusion may be at greater risk for

IARs. Patients with advanced Pompe disease may have compromised cardiac and respiratory function, which may predispose them to a higher risk of severe complications from IARs.

- Risk of Acute Cardiorespiratory Failure in Susceptible Patients
- Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function for whom fluid restriction is indicated may be at risk of serious exacerbation of their cardiac or respiratory status during cipaglucosidase alfa-atga infusion. More frequent monitoring of vitals should be performed during cipaglucosidase alfa-atga infusion in such patients

Resources:

https://www.micromedexsolutions.com/micromedex2/librarian/CS/A562A6/ND_PR/evidencexpert/ND_P/evidencexpert/DUPLICATIONSHIELDSYNC/AAA6C4/ND_PG/evidencexpert/ND_B/evidencexpert/ND_AppProduct/evidencexpert/ND_T/evidencexpert/PFActionId/evidencexpert.GoToDashboard?docId=933397&contentSetId=100&title=Avalglucosidase+Alfa-ngpt&servicesTitle=Avalglucosidase+Alfa-ngpt&brandName=Nexviazyme&UserMdxSearchTerm=Nexviazyme&=null#

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