Nexviazyme (avalglucosidase alfa-ngpt)
PAM – 045

Iowa Medicaid Program	Prior Authorization	**Effective Date**	01/01/2022
Revision Number	5	**Last Reviewed**	04/17/2026
Reviewed By	Medicaid Medical Director	**Next Review**	04/16/2027
Approved By	Medicaid Clinical Advisory Committee	**Approved Date**	07/15/2022

Overview

Medication: [1]	avalglucosidase alfa-ngpt
Brand Name:	Nexviazyme®
Pharmacologic Category:	Hydrolytic lysosomal glycogen-specific enzyme
FDA-Approved Indication(s):	Treatment of patients 1 year of age and older with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency)
How Supplied:	Single-dose vial containing 100 mg of lyophilized powder for reconstitution
Dosage and Administration:	Administered via intravenous (IV) infusion, based on body weight • Body weight ≥ 30 kg: dosage is 20 mg/kg every 2 weeks • Body weight < 30 kg: dosage is 40 mg/kg every 2 weeks
Benefit Category:	Medical

WARNING: HYPERSENSITIVITY REACTIONS, INFUSION-ASSOCIATED REACTIONS, and RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS

Hypersensitivity Reactions Including Anaphylaxis: Patients treated with Nexviazyme® have experienced life-threatening hypersensitivity reactions, including anaphylaxis. Appropriate medical monitoring and support measures, including cardiopulmonary resuscitation equipment, should be readily available during Nexviazyme® administration. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, discontinue Nexviazyme® immediately and initiate appropriate medical treatment. In patients with severe hypersensitivity reactions, a desensitization procedure to Nexviazyme® may be considered.

Infusion-Associated Reactions (IARs): Patients treated with Nexviazyme® have experienced severe IARs. If severe IARs occur, consider immediate discontinuation of Nexviazyme®, initiation of appropriate medical treatment, and the benefits and risks of readministering Nexviazyme® following severe IARs. Patients with an acute underlying illness at the time of Nexviazyme® 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 Nexviazyme® infusion. More frequent monitoring of vitals should be performed during infusion in such patients.

Descriptive Narrative

Acid alpha-glucosidase (GAA, also called acid maltase) deficiency (Pompe disease) was the first identified lysosomal storage disease. Pompe disease is an autosomal recessive disease resulting in deficient activity of GAA, the enzyme needed to degrade glycogen in lysosomes. Lysosomal GAA is needed to hydrolyze both alpha-1,4- and alpha-1,6-glucosidic linkages in the low pH environment of the lysosome. Deficiency of the enzyme leads to accumulation of glycogen in lysosomes and in the cytoplasm, resulting in tissue destruction.

The predicted incidence of late-onset Pompe disease is 1 in 57,000 (from a study in the Netherlands; based on carrier frequencies). The actual frequency of late-onset Pompe disease in the United States is not known, but studies investigating strategies for newborn screening in the U.S. found an incidence of 1 in 21,979 for the early- and late-onset forms combined.[2]

GAA deficiency has an infantile-onset ("classic") form as well as a late-onset (including juvenile and adult presentations) form. In the classic infantile form (Pompe disease), cardiomyopathy and muscular hypotonia are the cardinal features; in the juvenile and adult forms, involvement of skeletal muscles dominates the clinical picture.[3]

Infants with infantile-onset (“classic”) GAA deficiency typically present during the first few months of life. An early-onset "non-classic" phenotype is probably just an earlier presentation of the late-onset form of GAA deficiency, presenting with hypotonia without cardiomyopathy during the first one to two years of life.

Patients with late-onset GAA deficiency do not develop cardiomyopathy and may present at any age (age of onset is variable even in patients with identical variants). In late-onset GAA deficiency, the primary clinical finding is skeletal myopathy, with a more protracted course leading to respiratory failure.

Affected children usually present with delayed gross-motor development and progressive weakness in a limb-girdle distribution. Early involvement of the diaphragm is a common feature, and sleep-disordered breathing may occur. This usually leads to respiratory failure and death in the second or third decade of life.

Affected adults with late-onset GAA deficiency also present with progressive, proximal weakness in a limb-girdle distribution, particularly the hip flexors in the earliest stages of the disease. The weakness is accompanied by diaphragmatic involvement, leading to respiratory insufficiency early in the course of the disease.[4]

Guidelines

In 2014, the American College of Medical Genetics and Genomics published guidelines for the diagnosis and management of glycogen storage disease type 1. Consensus was developed in each area of diagnosis, treatment, and management. This practice guideline was developed prior to FDA approval of Nexviazyme® and so treatment with this medication is not addressed.

This management guideline specifically addresses evaluation and diagnosis across multiple organ systems (hepatic, kidney, gastrointestinal/nutrition, hematologic, cardiovascular, reproductive) involved in GSD I. Conditions to consider in the differential diagnosis stemming from presenting features and diagnostic algorithms are discussed. Aspects of diagnostic evaluation and nutritional and medical management, including care coordination, genetic counseling, hepatic and renal transplantation, and prenatal diagnosis, are also addressed.[5]

Criteria

Prior authorization is required.

Nexviazyme® is considered medically necessary when **ALL** of the following are met:

1. Diagnosis of non-infantile onset (late-onset) Pompe disease, confirmed either by an enzyme assay confirming low acid alpha-glucosidase (GAA) deficiency, or by 2 confirmed GAA gene mutations; **AND**
2. Member is 1 year of age or older; **AND**
3. Documentation of forced vital capacity (FVC) 30-85% of predicted value; **AND**
4. Member is able to walk 40 meters on a 6-minute walk test (6MWT) without assistive devices; **AND**
5. Nexviazyme® will **NOT** be prescribed in combination with Lumizyme®; **AND**
6. Prescribed by, or in consultation with, a medical geneticist or physician experienced in the treatment of Pompe disease; **AND**
7. Request meets one of the following (a or b):
    a. Regimen prescribed is based on actual body weight of member and does not exceed:
        i. Member weight is 30 kg or more: 20 mg/kg every 2 weeks;
        ii. Member weight is less than 30 kg: 40 mg/kg every 2 weeks; or,
    b. Regimen is supported by clinical practice guidelines. Supporting clinical documentation must be provided with any request for which regimen prescribed does not align with FDA-approved labeling.

Nexviazyme® is considered medically necessary for continuation of therapy when **ALL** of the following are met:

1. Member is currently receiving medication through the Iowa Medicaid benefit or has previously met initial approval criteria; **AND**
2. Documentation of positive clinical response to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, muscle strength), based on an evaluation by the prescribing provider; **AND**
3. Prescribed by, or in consultation with, a medical geneticist or physician experienced in the treatment of Pompe disease; **AND**
4. Request meets one of the following (a or b):
    a. Regimen prescribed is based on actual body weight of member and does not exceed:
        i. Member weight is 30 kg or more: 20 mg/kg every 2 weeks;
        ii. Member weight is less than 30 kg: 40 mg/kg every 2 weeks; or,
    b. Regimen is supported by clinical practice guidelines. Supporting clinical documentation must be provided with any request for which regimen prescribed does not align with FDA-approved labeling.

Approval Duration and Quantity Limits

	Initial Authorization	**Subsequent Authorization(s)**
Approval Duration	6 months	12 months
Quantity Limits	Member weight ≥ 30 kg: 20 mg/kg every 2 weeks Member weight < 30 kg: 40 mg/kg every 2 weeks	

Coding and Product Information

The following list(s) of codes and product information are provided for reference purposes only and may not be all inclusive. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment, nor does the exclusion of a code imply that its association to the HCPCS code is inappropriate.

HCPCS	**Description**
~~C9085~~	~~Injection, avalglucosidase alfa-ngpt, 4 mg *(effective 1/1/2022 – 3/31/2022)*~~
J0219	Injection, avalglucosidase alfa-ngpt, 4 mg *(effective 4/1/22)*

ICD-10	**Description**
E74.02	Pompe disease

NDC (Strength)	**Labeler**	**Dosage**	**Pkg Size**	**Pkg Qty**	**Units /Pkg**
58468-0426-01 (powder, 100 mg, in a single-dose vial)	Genzyme Corporation (58468)	4 mg	1	EA	25

Compliance

1. Should conflict exist between the policy and applicable statute, the applicable statute shall supersede.
2. Federal and State law, as well as contract language, including definitions and specific contract provisions or exclusions, take precedence over medical policy and must be considered first in determining eligibility for coverage.
3. Medical technology is constantly evolving, and Iowa Medicaid reserves the right to review and update medical policy on an annual or as-needed basis.

Medical necessity guidelines have been developed for determining coverage for member benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. Medical necessity guidelines are developed for selected physician-administered medications found to be safe and proven to be effective in a limited, defined population or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. Criteria are revised and updated annually, or more frequently if new evidence becomes available that suggests needed revisions.

References

[1] Nexviazyme® prescribing information (09/2023). Genzyme Corporation: Cambridge, MA. Available online: hcp.nexviazyme.com. Accessed January 28, 2026.

[2] Kaplan SL. Lysosomal acid alpha-glucosidase deficiency (Pompe disease, glycogen storage disease II, acid maltase deficiency). TePas E, ed. UpToDate. Waltham, MA: UpToDate Inc. www.uptodate.com. Accessed February 10, 2026.

[3] Online Mendelian Inheritance in Man, OMIM®. Johns Hopkins University: Baltimore, MD. MIM Number 232300. Last edited November 10, 2022. omim.org. Accessed March 12, 2024.

[4] Kaplan SL. Lysosomal acid alpha-glucosidase deficiency (Pompe disease, glycogen storage disease II, acid maltase deficiency). TePas E, ed. UpToDate. Waltham, MA: UpToDate Inc. www.uptodate.com. Accessed February 10, 2026.

[5] Kishnani PS, Austin SL, et al; American College of Medical Genetics and Genomics. Diagnosis and management of glycogen storage disease type I: a practice guideline of the American College of Medical Genetics and Genomics. Genet Med. 2014 Nov;16(11):e1. PMID: 25356975.

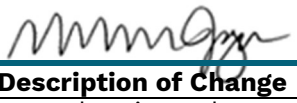
Development of utilization management criteria may also involve research into other state Medicaid programs, other payer policies, consultation with experts and review by the Medicaid Clinical Advisory Committee (CAC). These sources may not be referenced individually unless they are specifically published and are otherwise applicable to the criteria at issue.

Criteria Change History

Change Date	Changed By	Description of Change	Version
[mm/dd/yyyy]	CAC		
Signature			

Change Date	Changed By	Description of Change	Version
[mm/dd/yyyy]	CAC		
Signature			

Change Date	Changed By	Description of Change	Version
04/17/2026	CAC	Annual review. Updated predicted incidence in Descriptive Narrative. Updated references.	5
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/18/2025	CAC	Annual review. Updated references.	4
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/19/2024	CAC	Annual review. Changed review cycle from July to April to align with other enzyme deficiency policies. Updated Guidelines section. Added dosing information into criteria. Updated references.	3
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
07/21/2023	CAC	Annual review. Updated references.	2
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
07/15/2022	CAC	Criteria implementation.	1
Signature William (Bill) Jagiello, DO			

CAC = Medicaid Clinical Advisory Committee