

Clinical Policy: Avalglucosidase Alfa-ngpt (Nexviazyme)

Reference Number: ERX.SPA.426

Effective Date: 08.06.21

Last Review Date: 11.21

Line of Business: Commercial, Medicaid

[Revision Log](#)

See **Important Reminder** at the end of this policy for important regulatory and legal information.

Description

Avalglucosidase alfa-ngpt (Nexviazyme[™]) is a hydrolytic lysosomal glycogen-specific enzyme.

FDA Approved Indication(s)

Nexviazyme is indicated for the treatment of patients 1 year of age and older with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency).

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions[™] that Nexviazyme is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Pompe Disease (must meet all):

1. Diagnosis of late-onset Pompe disease confirmed by one of the following (a or b):
 - a. Enzyme assay confirming low GAA activity;
 - b. DNA testing;
2. Age \geq 1 year;
3. Nexviazyme is not prescribed concurrently with Lumizyme[®];
4. Dose does not exceed any of the following (a or b):
 - a. Members weighing \geq 30 kg: 20 mg/kg every 2 weeks;
 - b. Members weighing $<$ 30 kg: 40 mg/kg every 2 weeks.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

A. Pompe Disease (must meet all):

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's Pompe disease manifestation profile (*see Appendix D for examples*);
3. Nexviazyme is not prescribed concurrently with Lumizyme;
4. If request is for a dose increase, new dose does not exceed any of the following (a or b):
 - a. Members weighing \geq 30 kg: 20 mg/kg every 2 weeks;
 - b. Members weighing $<$ 30 kg: 40 mg/kg every 2 weeks.

Approval duration: 12 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

III. Diagnoses/Indications for which coverage is NOT authorized:

- A.** Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off-label use policy – ERX.PA.01 or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

6MWT: 6 minute walk test

FDA: Food and Drug Administration

GAA: acid alpha-glucosidase

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): none reported
- Boxed warning(s): severe hypersensitivity reactions; infusion-associated reactions; risk of acute cardiorespiratory failure in susceptible patients

Appendix D: Measures of Therapeutic Response

- Pompe disease manifests as a clinical spectrum that varies with respect to age at onset*, rate of disease progression, and extent of organ involvement. Patients can present with a variety of signs and symptoms, which can include cardiomegaly, cardiomyopathy, hypotonia, muscle weakness, respiratory distress (eventually requiring assisted ventilation), and skeletal muscle dysfunction.
- While there is not one generally applicable set of clinical criteria that can be used to determine appropriateness of continued therapy, clinical parameters that can indicate therapeutic response to NeoGAA include improved or maintained forced vital capacity, improved or maintained 6 minute walk test (6MWT) distance.

**Although infantile-onset disease typically presents in the first year of life, age of onset alone does not necessarily distinguish between infantile- and late-onset disease since juvenile-onset disease can present prior to 12 months of age.*

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Pompe disease	For patients weighing \geq 30 kg: 20 mg/kg every 2 weeks; For patients weighing < 30 kg: 40 mg/kg every 2 weeks	40 mg/kg/2 weeks

VI. Product Availability

Lyophilized powder in a single-dose vial: 100 mg

VII. References

1. Nexviazyme Prescribing Information. Cambridge, MA: Genzyme Corporation; August 2021. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2021/761194s000lbl.pdf. Accessed August 23, 2021.
2. Pena LDM, Barohn RJ, Byrne BJ, et al. Safety, tolerability, pharmacokinetics, pharmacodynamics, and exploratory efficacy of the novel enzyme replacement therapy avalglucosidase alfa (neoGAA) in treatment-naïve and alglucosidase alfa-treated patients with late-onset Pompe disease: A phase 1, open-label, multicenter, multinational, ascending dose study. *Neuromuscular Disorders* 2019;29:167-86.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	01.05.21	02.21
Drug is now FDA approved – criteria updated per FDA labeling: updated covered diagnosis to include only late-onset disease; added requirement for biochemical or genetic testing to confirm Pompe diagnosis, removed the requirement for an endocrinologist prescriber (aligns with Lumizyme policy), updated minimum age to 1 year old; references reviewed and updated.	08.23.21	11.21

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.

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