Clinical Policy Title:	avalglucosidase alfa-ngpt
Policy Number:	RxA.711
Drug(s) Applied:	Nexviazyme™
Original Policy Date:	12/07/2021
Last Review Date:	12/07/2021
Line of Business Policy Applies to:	All lines of business

Background

Avalglucosidase alfa-ngpt (Nexviazyme™) is a hydrolytic lysosomal glycogen-specific enzyme indicated for the treatment of patients 1 year of age and older with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency).

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
avalglucosidase alfa-ngpt (Nexviazyme™)	Pompe disease	Intravenous infusion for patients weighing: ≥ 30 kg, the dosage is 20 mg/kg (of actual body weight) every two weeks. < 30 kg, the recommended dosage is 40 mg/kg (of actual body weight) every two weeks.	Weighing ≥ 30 kg: 20 mg/kg/dose intravenously every 2 weeks. Weighing < 30 kg: 40 mg/kg/dose intravenously every 2 weeks.

Dosage Forms

- For injection: 100 mg of avalglucosidase alfa-ngpt as a lyophilized powder in a single-dose vial for reconstitution.

Clinical Policy

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria. The provision of provider samples does not guarantee coverage under the terms of the pharmacy benefit administered by RxAdvance. All criteria for initial approval must be met in order to obtain coverage.

I. Initial Approval Criteria

A. Late-Onset Pompe Disease (must meet all)**:**

1. Diagnosis of LOPD (lysosomal acid alpha-glucosidase [GAA] deficiency) as evidenced by one if the following (a or b):
   a. Enzyme assay showing a deficiency of acid alpha-glucosidase (GAA) activity in the blood, skin, or muscle. (GAA enzyme activity level ≤40% of normal mean value);
   b. Genetic testing showing a mutation in the GAA gene (presence of GAA enzyme deficiency or at least

one confirmed mutations in the GAA gene;
2. Age ≥ 1 year;
3. Prescribed by or in consultation with a metabolic specialist or biochemical geneticist;
4. Patient has measurable signs of Pompe disease, such as impairment in pulmonary function or motor weakness;
5. Documentation of baseline percent-predicted forced vital capacity (FVC) and 6-minute walk test (6MWT);
6. Member should NOT meet any of the following:
   a. Concomitant use of alglucosidase alfa (Lumizyme®);
   b. Previous failure of alglucosidase alfa (Lumizyme®);
   c. Patient is not able to ambulate 40 meters without stopping and without an assistive device;
   d. Patient has Pompe-specific cardiac hypertrophy;
   e. Patient requires invasive ventilation;
   f. Patient has a percent-predicted FVC of <30% or ≥85%.
7. Requested dose must meet one of the following (a or b):
   a. If the patient weighs ≥30 kg: 20 mg/kg;
   b. If the patient weighs <30 kg: 40 mg/kg.

Approval Duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

A. Pompe disease (must meet all):

1. Member is currently receiving medication that has been authorized by RxAdvance or the member has met initial approval criteria listed in this policy;
2. Member is responding positively to therapy, as evidenced by an improvement or stabilization in percent-predicted FVC and/or 6MWT;
3. Requested dose must meet one of the following:
   a. If the patient weighs ≥30 kg: 20 mg/kg;
   b. If the patient weighs <30 kg: 40 mg/kg.

Approval Duration
Commercial: 6 months
Medicaid: 6 months

III. Appendices

APPENDIX A: Abbreviation/Acronym Key
LOPD: Late-Onset Pompe Disease
GAA: acid alpha-glucosidase
IARs: Infusion-Associated Reactions
FVC: forced vital capacity
6MWT: 6 Minute Walk Test

APPENDIX B: Therapeutic Alternatives
Below are suggested therapeutic alternatives based on clinical guidance. Please check drug formulary for preferred agents and utilization management requirements.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
Lumizyme®	20 mg/kg/dose intravenously administered over about 4 hours every 2 weeks.	20 mg/kg/dose intravenously once every 2 weeks

Therapeutic alternatives are listed as generic (Brand name®) when the drug is available by both generic and brand, Brand name® when the drug is available by brand only and generic name when the drug is available by generic only.

APPENDIX C: Contraindications/Boxed Warnings

- Contraindication(s):
  - None reported.

- Boxed Warning(s):
  - Severe hypersensitivity reactions, infusion-associated reactions and risk of acute cardiorespiratory failure in susceptible patients.

APPENDIX D: General Information

- Hypersensitivity Reactions Including Anaphylaxis: Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available. If a severe hypersensitivity reaction occurs, Nexviazyme™ should be discontinued immediately and appropriate medical treatment should be initiated.
- Member should be administered antihistamines, antipyretics, and/or corticosteroids prior to avalglucosidase alfa-ngpt (Nexviazyme™) administration to reduce the risk of IARs;
- Infusion-Associated Reactions (IARs): If severe IARs occur, consider immediate discontinuation and initiation of appropriate medical treatment.
- 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, may be at risk of serious exacerbation of their cardiac or respiratory status during Nexviazyme™ infusion.

References

1. Nexviazyme™ prescribing information. Cambridge, MA: Genzyme Corporation; August 2021. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/nda/2021/761194Orig1s000lbl.pdf. Accessed October 5, 2021.
2. New Drug Review: Nexviazyme™. IPD Analytics. Available at: https://secure.ipdanalytics.com/User/Handler/ViewReport.ashx?type=RP&file=s3%3a%2f%2fipdanalytics%2fReports%2fIPD+Analytics+RxInsights_New+Drug+Review_Nexviazyme+_08+2021_1.pdf. Accessed October 5, 2021.
3. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.; 2019. Available at: http://www.clinicalpharmacology-ip.com. Accessed October 5, 2021.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	12/07/2021	12/07/2021