POLICY: Pompe Disease – Enzyme Replacement Therapy – Nexviazyme Utilization Management Medical Policy

- Nexviazyme® (avalglucosidase alfa-ngpt intravenous infusion – Genzyme)

EFFECTIVE DATE: 12/01/2021
REVIEW DATE: 09/16/2024

COVERAGE CRITERIA FOR: All UCare Plans

OVERVIEW

Nexviazyme, a hydrolytic lysosomal glycogen-specific recombinant human α-glucosidase enzyme, is indicated for **late-onset Pompe disease** (lysosomal acid α-glucosidase deficiency) in patients ≥ 1 year of age.[1]

Disease Overview

Pompe disease (glycogen storage disease type II, or acid maltase deficiency), is a rare lysosomal storage disorder characterized by a deficiency in acid α-glucosidase activity leading to the accumulation of glycogen, particularly in muscle.[2,3] The onset, progression, and severity of Pompe disease is variable. Infantile-onset Pompe disease usually manifests in the first few months of life and death often occurs in the first year of life, if left untreated.[2] Clinical manifestations of infantile-onset Pompe disease includes hypotonia, difficulty feeding, and cardiopulmonary failure.[4] Late-onset Pompe disease has a more variable clinical course and can manifest any time after 12 months of age.[3,4] Patients typically present with progressive muscle weakness which can progress to respiratory insufficiency. The diagnosis of Pompe disease is established by demonstrating decreased acid α-glucosidase activity in blood, fibroblasts, or muscle tissue; or by genetic testing.

POLICY STATEMENT

Prior Authorization is recommended for medical benefit coverage of Nexviazyme. Approval is recommended for those who meet the **Criteria** and **Dosing** for the listed indication. Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Nexviazyme as well as the monitoring required for adverse events and long-term efficacy, approval requires Nexviazyme to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Nexviazyme is recommended in those who meet the following criteria:

FDA-Approved Indication

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1. **Acid Alpha-Glucosidase Deficiency (Pompe Disease).** Approve for 1 year if the patient meets ALL of the following (A, B, C, and D):
   - **A)** Patient is ≥ 1 year of age; AND
   - **B)** Patient has late-onset acid alpha-glucosidase deficiency (late-onset Pompe disease); AND
   - **C)** The diagnosis is established by ONE of the following (i or ii):
     - **i.** Patient has a laboratory test demonstrating deficient acid alpha-glucosidase activity in blood, fibroblasts, or muscle tissue; OR
     - **ii.** Patient has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic acid alpha-glucosidase (GAA) gene variants; AND
   - **D)** The medication is prescribed by or in consultation with a geneticist, neurologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

   Dosing. Approve ONE of the following dosing regimens (A or B):
   - **A)** Patient ≥ 30 kg: Dose is 20 mg/kg administered by intravenous infusion once every 2 weeks; OR
   - **B)** Patient < 30 kg: Dose is 40 mg/kg administered by intravenous infusion once every 2 weeks.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Nexviazyme is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

REFERENCES

1. Nexviazyme ® intravenous infusion [prescribing information]. Cambridge, MA: Genzyme; April 2023.
2. Chien YH, Hwu WL, Lee NC. Pompe disease: Early diagnosis and early treatment make a difference. *Pediatr Neonatol*. 2013;54:219-227.
3. Llerena Junior JC, Nascimento OJM, Oliveira ASB, et al. Guidelines for the diagnosis, treatment and clinical monitoring of patients with juvenile and adult Pompe disease. *Arq Neuropsiquiatr*. 2016;74:166-176.
4. Cupler EJ, Berger KI, Leshner RT, et al. Consensus treatment recommendations for late-onset Pompe disease. *Muscle Nerve*. 2012;45:319-333.

HISTORY

Type of Revision	Summary of Changes	Review Date
Annual Revision	No criteria changes.	08/23/2023
Update	10/04/2023: No criteria changes. Policy name changed from Enzyme Replacement Therapy – Nexviazyme to Pompe Disease – Enzyme Replacement Therapy – Nexviazyme.	NA
Early Annual Revision	**Acid Alpha-Glucosidase Deficiency (Pompe Disease):** Confirmation of a genetic mutation in the acid alpha-glucosidase gene was rephrased to more specifically state, "genetic test demonstrating biallelic pathogenic or likely pathogenic acid alpha-glucosidase gene variants".	05/08/2024
UCare P&T Review	Policy reviewed and approved by UCare P&T committee. Annual review process	09/16/2024