

PHARMACY POLICY STATEMENT

Marketplace

DRUG NAME	Nexviazyme (avalglucosidase alfa-ngp)
BILLING CODE	J0219
BENEFIT TYPE	Medical
SITE OF SERVICE ALLOWED	Home/Office/Outpatient
STATUS	Prior Authorization Required

Nexviazyme is an enzyme replacement therapy for the treatment of Pompe disease, also known as acid alpha-glucosidase (GAA) deficiency or glycogen storage disease type II. Pompe disease is a rare, genetic lysosomal storage disorder that results in the buildup of glycogen in cell lysosomes causing serious and life-threatening muscle damage and weakness. Nexviazyme provides an exogenous source of the deficient GAA enzyme to cleave glycogen and reduce its accumulation. In the COMET trial, Nexviazyme was found to be non-inferior to Lumizyme.

Pompe disease can be broadly classified as infantile-onset within the first few months of life (IOPD) or late-onset beyond infancy (LOPD). Classic IOPD is rapidly progressive with severe cardiomyopathy. Non-classic IOPD progresses slower with less severe cardiomyopathy. LOPD does not typically present with cardiomyopathy and has more variable symptoms, especially skeletal muscle weakness. Nexviazyme is only indicated to treat late-onset Pompe disease.

Nexviazyme (avalglucosidase alfa-ngp) will be considered for coverage when the following criteria are met:

Pompe disease (acid α -glucosidase [GAA] deficiency)

For **initial** authorization:

1. Member is at least 1 year of age; AND
2. Medication must be prescribed by or in consultation with a geneticist, neurologist, pulmonologist, or metabolic specialist; AND
3. Member has a diagnosis of **late onset** Pompe disease confirmed by an enzyme activity assay showing GAA deficiency (2% to 40% of normal); AND
4. Molecular genetic testing shows pathogenic mutation of the GAA gene; AND
5. Member must show signs or symptoms (i.e., motor weakness, reduced respiratory parameters).
6. **Dosage allowed/Quantity limit:**
 Actual body weight 30 kg or greater: 20 mg/kg every 2 weeks
 Actual body weight less than 30 kg: 40mg/kg every 2 weeks

If all the above requirements are met, the medication will be approved for 12 months.

For **reauthorization**:

1. Chart notes must document positive clinical response such as improved or stabilized respiratory muscle strength (i.e., forced vital capacity (FVC)) or functional endurance (e.g., 6-minute walk test).

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Nexviazyme (avalglucosidase alfa-ngp) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
08/20/2021	New policy for Nexviazyme created.
05/24/2022	Updated J code.

References:

1. Nexviazyme [package insert]. Genzyme Corporation; 2021.
2. Tarnopolsky M, Katzberg H, Petrof BJ, et al. Pompe Disease: Diagnosis and Management. Evidence-Based Guidelines from a Canadian Expert Panel. *Can J Neurol Sci.* 2016;43(4):472-485. doi:10.1017/cjn.2016.37
3. Burton BK, Kronn DF, Hwu WL, Kishnani PS; Pompe Disease Newborn Screening Working Group. The Initial Evaluation of Patients After Positive Newborn Screening: Recommended Algorithms Leading to a Confirmed Diagnosis of Pompe Disease. *Pediatrics.* 2017;140(Suppl 1):S14-S23. doi:10.1542/peds.2016-0280D
4. Kronn DF, Day-Salvatore D, Hwu WL, et al. Management of Confirmed Newborn-Screened Patients With Pompe Disease Across the Disease Spectrum. *Pediatrics.* 2017;140(Suppl 1):S24-S45. doi:10.1542/peds.2016-0280E
5. Study to Compare the Efficacy and Safety of Enzyme Replacement Therapies Avalglucosidase Alfa and Alglucosidase Alfa Administered Every Other Week in Patients With Late-onset Pompe Disease Who Have Not Been Previously Treated for Pompe Disease (COMET). ClinicalTrials.gov Identifier: NCT02782741. Updated April 8, 2021. Accessed August 23, 2021. <https://clinicaltrials.gov/ct2/show/NCT02782741>
6. van der Ploeg AT, Kruijshaar ME, Toscano A, et al. European consensus for starting and stopping enzyme replacement therapy in adult patients with Pompe disease: a 10-year experience. *Eur J Neurol.* 2017;24(6):768-e31. doi:10.1111/ene.13285
7. Wang RY, Bodamer OA, Watson MS, Wilcox WR; ACMG Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genet Med.* 2011;13(5):457-484. doi:10.1097/GIM.0b013e318211a7e1
8. Cupler EJ, Berger KI, Leshner RT, et al. Consensus treatment recommendations for late-onset Pompe disease. *Muscle Nerve.* 2012;45(3):319-333. doi:10.1002/mus.22329
9. Kushlaf H, Attarian S, Borges JL, et al. Efficacy and Safety Results of the Avalglucosidase alfa Phase 3 COMET Trial in Late-Onset Pompe Disease Patients (4195). *Neurology.* 2021;96(15 Supplement). https://n.neurology.org/content/96/15_Supplement/4195/tab-article-info

Effective date: 10/01/2022

Revised date: 05/24/2022