BlueCross BlueShield Federal Employee Program

NEXVIAZYME
(avalglucosidase alfa-ngpt)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Nexviazyme (avalglucosidase alfa-ngpt) is indicated for late-onset Pompe disease, a rare genetic disorder. In Pompe disease, a gene mutation prevents the body from making an enzyme or making enough of the enzyme called acid alpha-glucosidase (GAA), necessary for proper muscle function. GAA is used by the heart and muscle cells to convert stored glycogen into energy. Without sufficient enzyme action, glycogen builds up in the cells, ultimately weakening the heart and other muscles. Infusion of Nexviazyme replaces the deficient GAA, reducing the accumulated glycogen in the body (1).

Regulatory Status

FDA-approved indication: 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) (1).

Nexviazyme has a boxed warning for severe hypersensitivity reactions, including anaphylaxis, infusion-associated reactions, and risk of cardiorespiratory failure in susceptible patients. In severe hypersensitivity reactions (e.g., anaphylaxis), Nexviazyme should be discontinued immediately and appropriate treatment initiated. Infusion-associated reactions (IARs) have also been reported to occur at any time during infusion, up to a few hours after the infusion has concluded. In severe IARs, immediate discontinuation of Nexviazyme and appropriate care should be provided. In mild to moderate IARs, rechallenge with slower infusion rates or lower doses have been shown to reduce symptoms. Patients with advanced Pompe disease may have compromised heart and lung function and are at serious risk of decompensation during Nexviazyme infusion. Vitals should be monitored more frequently in this population, and some patients may require prolonged observation times (1).

Patients with Pompe disease may have compromised cardiac function, including issues such as cardiomyopathy, heart failure, and arrhythmia. Prior to Nexviazyme infusion, an echocardiogram may be helpful to assess for cardiomyopathy or cardiac hypertrophy and a 12 lead EKG can assist in excluding arrhythmia (2).

The safety and effectiveness of Nexviazyme in pediatric patients less than 1 year of age have not been established (1).

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Summary

Nexviazyme (avalglucosidase alfa-ngpt) is a lysosomal glycogen-specific enzyme indicated for patients 1 year of age and older with late-onset Pompe disease (acid α-glucosidase (GAA) deficiency). Nexviazyme has a boxed warning that hypersensitivity reactions, infusion-associated reactions and cardiorespiratory failure in susceptible patients has been observed during and after infusions. Appropriate medical support should be available during infusion and some patients may require prolonged observation time after infusion has concluded. The safety and effectiveness of Nexviazyme in pediatric patients less than 1 year of age have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Nexviazyme while maintaining optimal therapeutic outcomes.

References

1. Nexviazyme [package insert]. Cambridge, MA: Genzyme Corporation; September 2023.
2. Kishnani, P. S., Steiner, R. D., Bali, D., Berger, K., Byrne, B. J., Case, L. E., Crowley, J. F., Downs, S., Howell, R. R., Kravitz, R. M., Mackey, J., Marsden, D., Martins, A. M., Millington, D. S., Nicolino, M., O'Grady, G., Patterson, M. C., Rapoport, D. M., Slonim, A., Watson, M. S. (2006, May). *Pompe disease diagnosis and management guideline*. Genetics in medicine : official journal of the American College of Medical Genetics. Retrieved from https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3110959/.