SPECIALTY GUIDELINE MANAGEMENT

Myozyme (alglucosidase alfa)

POLICY

A. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication

- Myozyme is indicated for patients with Pompe disease (acid alpha-glucosidase [GAA] deficiency). Myozyme has been shown to improve ventilator-free survival in patients with infantile-onset Pompe disease as compared to an untreated historical control, whereas use of Myozyme in patients with other forms of Pompe disease has not been adequately studied to assure safety and efficacy.

All other indications are considered experimental/investigational and are not a covered benefit.

B. REQUIRED DOCUMENTATION

The following documentation is required to initiate a prior authorization review:

- Results of enzyme assay or DNA testing supporting the diagnosis

C. CRITERIA FOR APPROVAL

1. Pompe Disease

Indefinite authorization may be granted to members with a diagnosis of Pompe disease which was confirmed by an enzyme assay demonstrating a deficiency of acid alpha-glucosidase enzyme activity or by DNA testing

D. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must meet ALL initial authorization criteria.

E. DOSAGE AND ADMINISTRATION

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

REFERENCES

1. Myozyme [package insert]. Cambridge, MA: Genzyme Corporation; May 2014.
2. Kishnani PS, Howell RR. Pompe disease in infants and children. *J Pediatr.* 2004;144(5 Suppl):S35-S43.
3. Kishnani PS, Steiner RD, Bali D, et al, ACMG Work Group on Management of Pompe Disease. Pompe disease diagnosis and management guideline. *Genet Med.* 2006;8(5):267-268.
4. Kishnani PS, Corzo D, Nicolino M, et al. Recombinant human acid [alpha]-glucosidase: major clinical benefits in infantile-onset Pompe disease. *Neurology*. 2007;68(2):99-109.
5. van der Ploeg AT, Clemens PR, Corzo D, et al. A randomized study of alglucosidase alfa in late-onset Pompe's disease. *N Engl J Med*. 2010;362(15):1396-1406.