UnitedHealthcare Community Plan

Clinical Pharmacy Program Guidelines for Myozyme, Lumizyme -ARIZONA

Program	Prior Authorization
Medication	Myozyme, Lumizyme (alglucosidase alfa)

1. Background:

Lumizyme is indicated for patients with Pompe disease [acid alpha-glucosidase (GAA) deficiency].

Myozyme is indicated for use in patients with Pompe disease (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.

2. Coverage Criteria:

A. Authorization

1. Lumizyme or Myozyme will be approved based on the following:
    a. Diagnosis of Pompe disease (GAA deficiency)

Authorization will be issued for 12 months.

There are different tests available for diagnosis of Pompe disease. [3] The clinical diagnosis is traditionally confirmed by the virtual absence (infantile-onset) or markedly reduced (late onset) GAA activity in tissues such as cultured fibroblasts from skin biopsy, muscle biopsy, purified lymphocytes, mononuclear cells and lymphoid cell lines. Historically, GAA enzyme measurement is most reliably performed in cultured fibroblasts or muscle due to the possibility of alternate isoenzyme activities making disease in white cell assays. New methods have now been developed that assay GAA activity in dried blood spot (DBS) extracts. DBS can be conveniently collected by the heel-or finger-stick method and shipped from locations remote from the analytical center. [4] Diagnosis of Pompe disease should be confirmed by ordering one of the following tests to measure acid alpha-glucosidase (GAA) enzyme activity: [5] (1) Dried blood spot (blood draw, heel prick, or finger stick): turnaround time for results: 2-10 days (2) Lymphocytes (blood draw): turnaround time for results: 7-10 days (3) Mixed lymphocytes (blood draw): turnaround time for results: 7-10 days (4) Fibroblasts (skin biopsy): turnaround time for results: 4-6 weeks (5) Muscle tissue (muscle biopsy): turnaround time for results: 1-4 weeks.

3. References:

1. Myozyme Prescribing Information. Genzyme Corporation, May 2014.
2. Lumizyme Prescribing Information. Genzyme Corporation, August 2014.
3. Per clinical consultation with geneticist, November 10, 2010.
4. Kishani PS, Steiner RD, Bali, D. ACMG Practice Guideline. Pompe disease diagnosis and management guideline. Genet Med. 2006;8(5):267-88.
5. Diagnosing Pompe Disease (also known as Acid Maltase Deficiency). Available at: http://www.pompe.com/~/media/Pompe/Files/en/PDF/POMPUSP141_LabList.pdf. Accessed August 10, 2014.

Program	Program type – Prior Authorization
Change Control	
Date	Change
August 2017	New policy specific to Arizona