Medicare Advantage Medical Benefit Drug Policy

BlueCross BlueShield Nebraska

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Effective Date: 10/08/2020

Lumizyme® (alglucosidase alfa)

FDA approval: May 24, 2010
HCPCS: J0221
Benefit: Medical

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

A. Coverage of the requested drug is provided when all the following are met:
   a. FDA approved indication
   b. FDA approved age
   c. Prescribed by or in consultation with a geneticist or metabolic specialist
   d. Confirmation of diagnosis by serum assay showing a decrease of acid α-glucosidase activity followed by genetic testing showing a mutation in the GAA gene
   e. In late-onset disease, symptomatic manifestations of the disease must be present, including but not limited to, progressive muscle weakness, respiratory failure, frequent upper airway infections, orthopnea, sleep apnea, and/or morning headaches (must not present with only cardiac hypertrophy)
   f. Trial and failure, contraindication, OR intolerance to the preferred drugs as listed in BCBSNE Part B drugs prior authorization list

B. Quantity Limitations, Authorization Period and Renewal Criteria
   a. Quantity Limits: Align with FDA recommended dosing
   b. Authorization Period: 1 year at a time
   c. Renewal Criteria: Clinical documentation must be provided to confirm that current criteria are met and that the medication is providing clinical benefit

***Note: Coverage may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at http://www.cms.hhs.gov/. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Therapeutic considerations:

A. FDA approved indication / Diagnosis

**Please refer to most recent prescribing information.*

B. Background Information

a. Pompe disease (PD) is an autosomal recessive lysosomal storage disorder caused by a mutation on the GAA gene. It is characterized by lysosomal accumulation of undegraded glycogen due to a deficiency or insufficient activity of the enzyme acid α-glucosidase. All patients with PD have variable but progressive, skeletal, heart, and smooth muscles issues with resulting organ damage and ultimate organ failure. The disease is classified as either infantile-onset or late-onset. Patients with infantile-onset PD present around 3 months of age with progressive left ventricular hypertrophy and generalized muscular hypotonia and typically die within the first year of life because of cardiorespiratory failure. They may also experience macroglossia, hepatosplenomegaly, and feeding difficulties. Late-onset PD can present at any age and is characterized by a slowly progressive myopathy predominantly involving skeletal muscle. Symptoms include progressive muscle weakness of the proximal lower limbs and the paraspinal muscles, respiratory failure, frequent upper airway infections, orthopnea, sleep apnea, and morning headaches.

b. The American College of Medical Genetics 2011 guidelines state Pompe disease is confirmed through serum assay showing a decrease of acid α-glucosidase enzyme activity. Once shown the patient has a decrease in enzyme activity, a genetic test should be performed which should show a mutation in the GAA gene. Both tests must be demonstrative of disease for the diagnosis to be confirmed.

c. Enzyme replacement therapy (ERT) is the standard of care in PD. Lumizyme is the only ERT approved for use in pediatric and adult patients with Pompe disease.

d. Initiation of therapy should begin at the time of diagnosis for patients with infantile disease. ERT can be held in late-onset patients with patients being observed every 6 – 12 months until the time the monitoring physician feels it is appropriate to start therapy. Studies have been conducted assessing the efficacy of enzyme replacement therapy on cardiac symptoms in late-onset patients. Cardiovascular parameters were not impacted by use of ERT and therefore should not be the only presenting symptom when therapy is started.

C. Efficacy

**Please refer to most recent prescribing information.*

D. Medication Safety Considerations

**Please refer to most recent prescribing information.*

E. Dosing and administration

**Please refer to most recent prescribing information.*

F. How supplied

**Please refer to most recent prescribing information.*

References:

1. Lumizyme [prescribing information]. Cambridge, MA: Genzyme Corporation; February 2020.
2. Wang RW, Bodamer OA, Watson MS, et al. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. Genetic Med. 2011 May; 13 (3): 457 – 84.

3. Cupler EJ, Berger KI, Leshner RT, et al. Consensus treatment recommendations for late-onset pompe disease. Muscle Nerve. 2012; 45: 319 – 33.
4. Kishnani PS, Steiner RD, Bali D, et al. Pompe disease diagnosis and management guideline. Genetic Med. 2006 May; 8 (5): 267 – 88.
5. Forsha D, Li JS, Smith B, et al. Cardiovascular abnormalities in late onset pompe disease and response to enzyme replacement therapy. Genet Med. 2011 Jul; 13 (7): 625 – 31.

Policy History		
#	Date	Change Description
1.0	10/08/2020	Medical Policy established

** The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or http://dailymed.nlm.nih.gov/dailymed/index.cfm.*