Lumizyme 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. Lumizyme replaces the deficient GAA enzyme to reduce the glycogen accumulation.

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.

Lumizyme (alglucosidase alfa) will be considered for coverage when the following criteria are met:

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

For **initial** authorization:
1. Medication must be prescribed by or in consultation with a geneticist, cardiologist, neurologist, pulmonologist, or metabolic specialist; AND
2. Member has a diagnosis of Pompe disease confirmed by an enzyme activity assay showing GAA deficiency (0% to 40% of normal); AND
3. Molecular genetic testing shows pathogenic mutation of the GAA gene; AND
4. Members with late onset Pompe disease must show signs or symptoms (i.e., motor weakness, reduced respiratory parameters).
5. **Dosage allowed/Quantity limit:** 20 mg/kg IV infusion 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 motor function or ambulation, pulmonary function, or cardiomyopathy.

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

CareSource considers Lumizyme (alglucosidase alfa) 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.
New policy for Lumizyme created.

Annual review; added OAC reference.

References:


Effective date: 04/01/2023
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