Protocol for Lumizyme® (alglucosidase alfa)
Approved July 2021

Background:
Pompe disease is a rare, autosomal recessive disorder caused by deficiency of the glycogen-degrading lysosomal enzyme, acid alpha-glucosidase (GAA). Late-onset Pompe disease is a multisystem condition, with a heterogeneous clinical presentation that mimics other neuromuscular disorders.

Lumizyme *(alglucosidase alfa) is a hydrolytic lysosomal glycogenspecific enzyme indicated for patients with Pompe disease or GAA deficiency.*

Criteria for approval:

1. Patient has a diagnosis of infantile-onset Pompe disease as confirmed by ONE of the following:
    a. Absence or deficiency (< 1% of the lab specific normal mean) acid alpha-glucosidase deficiency activity in fibroblasts, lymphocytes, or muscle; OR
    b. Increased lysosomal glycogen; OR
    c. Molecular genetic testing for deletion or mutation in the GAA gene; OR
2. Patient has a diagnosis of late-onset (non-infantile) Pompe disease as confirmed by ONE of the following:
    a. Absence or deficiency (< 40% of the lab specific normal mean) GAA activity in lymphocytes, fibroblasts, or muscle; OR
    b. Increased lysosomal glycogen; OR
    c. Molecular genetic testing for deletion or mutation in the GAA gene
3. Medication is prescribed by or in consultation with a geneticist, metabolic disorders specialist, or an expert in the disease state
4. Patient's weight must be provided and have been taken within the last four weeks to ensure accurate dosing
5. Patient does not have any contraindication(s) to the requested medication
6. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Initial Approval: 6 months

Continuation of therapy:

1. Patient has experienced a positive clinical response to Lumizyme therapy (e.g., improved cardiac/respiratory function etc.)
2. For dose increase requests, weight must be received
3. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service,

Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Renewal Approval: 6 months

Note: Lumizyme has a black box warning:

WARNING: RISK OF ANAPHYLAXIS, HYPERSENSITIVITY AND IMMUNE-MEDIATED REACTIONS, AND RISK OF CARDIORESPIRATORY FAILURE

Life-threatening anaphylactic reactions and severe hypersensitivity reactions have occurred in some patients during and after alglucosidase alfa infusions. Immune mediated reactions presenting as proteinuria, nephrotic syndrome, and necrotizing skin lesions have occurred in some patients following alglucosidase alfa treatment. Closely observe patients during and after alglucosidase alfa administration and be prepared to manage anaphylaxis and hypersensitivity reactions. Inform patients of the signs and symptoms of anaphylaxis, hypersensitivity reactions, and immune mediated reactions and have them seek immediate medical care should signs and symptoms occur.

Infantile-onset Pompe disease patients with compromised cardiac or respiratory function may be at risk of serious acute exacerbation of their cardiac or respiratory compromise due to fluid overload and require additional monitoring.

References:

1. Lumizyme [Product information]. Genzyme Corporation . Cambridge MA 02142. 2/2020
2. Tarnopolsky M et al. Pompe Disease: Diagnosis and Management. Evidence-Based Guidelines from a Canadian Expert Panel. Can J Neurol Sci. 2016; 43:472-485. Volume 43, No. 4 – July 2016
3. Clinical Pharmacology (online database). Tampa FL: Gold Standard Inc.: 2019. Updated periodically
4. Cupler EJ, Berger KI et al. Consensus Treatment Recommendations for Late-Onset Pompe Disease. AANEM Muscle Nerve 45: 319–333, 2012
5. Bali D et al. Pompe disease diagnosis and management guideline. Genet Med. 2006 May; 8(5): 267–288