

RX.PA.034.MPC Specialty Enzymes (Lumizyme and Fabrazyme)

The purpose of this policy is to define the prior authorization process for the following specialty enzymes: Lumizyme (alglucosidase alfa) and Fabrazyme (agalsidase beta).

- Lumizyme (alglucosidase alfa) is indicated for patients with Pompe disease. Lumizyme consists of the human enzyme acid alpha-glucosidase (GAA) and are intended for intravenous infusion.
- Fabrazyme (agalsidase beta) is a recombinant human enzyme indicated for use in patients with Fabry disease. Agalsidase beta (Fabrazyme) reduces globotriasylceramide (GL-3) deposition in capillary endothelium of the kidney and certain other cell types.

DEFINITIONS

Mucopolysaccharidosis I – a rare, autosomal recessive genetic disease caused by a defect in the gene coding for the lysosomal enzyme alpha-L-iduronidase resulting in inability to produce sufficient amounts of the enzyme

Hunter Syndrome – a serious progressive genetic disorder caused by a deficiency or absence of the lysosomal enzyme (iduronate-2-sulfatase) required for the degradation of glycosaminoglycans (GAG) resulting in accumulation of GAG in cells throughout the body. Hunter Syndrome affects males almost exclusively.

Mucopolysaccharidosis VI – a progressive lysosomal storage disorder caused by a deficiency in the arylsulfatase B enzyme causing retention of glycosaminoglycans leading to multisystemic organ damage

Pompe Disease – A genetic absence or deficiency of acid alpha-glucosidase (GAA) resulting in build-up of glycogen in the cardiac and skeletal muscles, and in hepatic tissue. This results in the development of cardiomyopathy, progressive muscle weakness, and impairment of respiratory function.

Fabry Disease – a rare genetic disorder caused by a defect in the gene for the lysosomal enzyme alpha-galactosidase resulting in inability or diminished ability to catabolize certain lipids. These lipids then accumulate in many cell-types throughout the body.

Specialty Enzymes (Lumizyme and Fabrazyme)

POLICY NUMBER: RX.PA.034.MPC

REVISION DATE: 02/2020

PAGE NUMBER: 2 of 3

Globotriaosylceramide – a type of glycolipid compound that accumulates in blood vessel walls of people with Fabry disease

PROCEDURE

Initial Authorization Criteria:

Must meet all of the criteria listed below under each respective drug.

Lumizyme (alglucosidase alfa)

- Must be prescribed by or in consultation with a physician who specializes in the treatment of inherited metabolic disorders or a neurologist
- Must have a confirmed diagnosis of alpha glucosidase deficiency (Pompe disease)
 - Diagnosis must be confirmed through GAA enzyme assay (from blood, skin fibroblasts, lymphocytes, or muscle) and/or identification of GAA gene mutation

Fabrazyme (agalsidase beta) and Galafold (migalastat)

- Must be prescribed by or in consultation with a provider who specializes in the treatment of inherited metabolic disorders
- Must have a diagnosis of Fabry disease confirmed by one of the following:
 - Genetic test confirming mutation of galactosidase alpha (GLA) gene
 - Biopsy of tissue or organ (such as kidney) showing intracellular globotriaosylceramide (Gb3) inclusion
 - Confirmed family history of Fabry disease and familial mutation has been identified
 - Male members only may also have their diagnosis confirmed by an Alpha-galactosidase A (alpha-Gal A) enzyme activity <3%

Reauthorization Criteria:

All prior authorization renewals are reviewed on an annual basis to determine the Medical Necessity for continuation of therapy. Authorization may be extended at 12-month intervals based upon chart documentation from the prescriber that the member's condition has improved based upon the prescriber's assessment while on therapy.

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 1 year
Reauthorization	Same as initial

Specialty Enzymes (Lumizyme and Fabrazyme)

POLICY NUMBER: RX.PA.034.MPC

REVISION DATE: 02/2020

PAGE NUMBER: 3 of 3

If the established criteria are not met, the request is referred to a Medical Director for review.

Codes: J Code(s)

Code	Description
J0180	Injection, agalsidase beta, 1 mg
J0221	Injection, alglucosidase alfa, (lumizyme), 10 mgf

REFERENCES

1. Aldurazyme [package insert]. BioMatin/Genzyme LLC. Novato CA, April 2008.
2. Elaprase [package insert]. Shire Human Genetic Therapies Inc. Cambridge MA, October 2007.
3. Naglazyme [package insert]. BioMarin Pharmaceuticals. Novato CA, June 2005.
4. Lumizyme [package insert]. Genzyme Corp. Cambridge, MA, September 2014.
5. American Association of Neuromuscular & Electrodiagnostic Medicine. Diagnostic criteria for late-onset (childhood and adult) Pompe disease. Muscle Nerve 2009;40:149-160
6. Kishnani PS, Steiner RD, Bali D, et al. Pompe disease diagnosis and management guideline. Genet Med 2006;8(5):267-288
7. Kishnani PS, Amartino HM, Lindberg C, et al. Methods of diagnosis of patients with Pompe disease: data from the Pompe registry. Mol Genet Metab 2014; <http://dx.doi.org/10.1016/j.ymgme.2014.07.014>
8. Fabrazyme [package insert]. Genzyme Corporation. Cambridge MA, December 2008.
9. Galafold™ [Prescribing Information]. Cranbury, NJ: Amicus Therapeutics; August 2018.