

## **RX.PA.021.MPC Mucopolysaccharidosis (MPS) Enzyme Replacement Therapy**

The purpose of this policy is to define the prior authorization process for the Mucopolysaccharidosis enzyme replacement treatments:

- Aldurazyme (laronidase)
- Naglazyme (galsulfase)
- Elapraxe (idursulfase)
- Vimizim (elosulfase alfa)
- Mepsevii (vestronidase alfa-vjbc)

Aldurazyme® (laronidase) is a polymorphic variant of a human enzyme indicated for patients with Hurler and Hurler–Scheie forms of Mucopolysaccharidosis I (MPS I) and for patients with the Scheie form who have moderate to severe symptoms.

Naglazyme® (galsulfase) is a variant form of a polymorphic human enzyme indicated for patients with Mucopolysaccharidosis VI (MPS VI). Naglazyme® (galsulfase) has been shown to improve walking and stair-climbing capacity.

Elapraxe® (idursulfase) is a purified form of a lysosomal enzyme (iduronate-2-sulfatase) indicated for patients with Hunter Syndrome (Mucopolysaccharidosis II, MPS II). Elapraxe® (idursulfase) has been shown to improve walking capacity in these patients.

Vimizim® (elosulfase alfa) is indicated for the treatment of mucopolysaccharidosis type IVA (also known as MPS IVA, Morquio A, and Morquio A syndrome).

Mepsevii (vestronidase alfa-vjbc) is a recombinant human lysosomal beta glucuronidase indicated in pediatric and adult patients for the treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome)

### **DEFINITIONS**

**DBS** – dried blood spot

**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 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

**Mucopolysaccharidosis type IVA Morquio A syndrome** – autosomal recessive lysosomal storage disorder result from a deficiency in GALNS activity, which results in an accumulation of keratin sulfate and chondroitin-6-sulfate in the lysosome. This accumulation leads to impaired cellular function, which causes short stature, skeletal dysplasia, bone deformity, as well as reduced visual, auditory, digestive, cardiovascular and respiratory function.

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

**N-acetylgalactosamine-6-sulfate sulfatase (GALNS)** – lysosomal enzyme responsible for degrading glycosaminoglycans keratin sulfate and chondroitin-6-sulfate

The drugs, mucopolysaccharidosis enzyme replacement agents, are subject to the prior authorization process.

## PROCEDURE

### A. Initial Authorization Criteria:

*Must meet all of the criteria listed below:*

- Must be prescribed by or in consultation with a physician who specializes in the treatment of inherited metabolic disorders
- Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling
- Must have the appropriate diagnosis for the requested product:
  - **Aldurazyme:** Must have a confirmed diagnosis of Mucopolysaccharidosis, Type I (Hurler and Hurler-Scheie forms) or the Scheie form with moderate-to-severe symptoms
  - **Naglazyme:** Must have a confirmed diagnosis of Mucopolysaccharidosis VI (Maroteaux-Lamy syndrome)
  - **Elaprase:** Must have a confirmed diagnosis of Hunter syndrome (Mucopolysaccharidosis type II, MPS II)
  - **Vimizim:** Must have a diagnosis of Mucopolysaccharidosis type Morquio

A syndrome. Diagnosis must be confirmed by ONE of the following methods:

- GALNS enzyme activity assay (from leukocytes or fibroblasts) demonstrating a deficiency in GALNS activity. Documentation of laboratory result (including laboratory reference range) is required.
- GALNS gene molecular analysis demonstrating mutation in both GALNS alleles. Documentation of laboratory result of GAA gene mutation analysis is required.
- **Mepsevii:** Must have a confirmed diagnosis of mucopolysaccharidosis VII (MPS VII, Sly syndrome)

**B. Must be prescribed at a dose within the manufacturer’s dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.**

**C. Mucopolysaccharidosis treatments will be considered investigational or experimental for any other use and will not be covered.**

**D. 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 1-year 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

If the established criteria are not met, the request is referred to a Medical Director for review, if required for the plan and level of request.

**HCPCS Codes:**

Code:	Description:
J1322	Injection, elosulfase alfa, 1 mg
J1458	Injection, galsulfase, 1 mg
J1743	Injection, idursulfase, 1 mg
J1931	Injection, laronidase, 0.1 mg

**REFERENCES**

1. Aldurazyme [package insert]. BioMatin/Genzyme LLC. Novato CA, April 2008.
2. Vimizim [prescribing information]. Novato, CA: BioMarin Pharmaceutical, Inc.; 2014
3. Vimizim [AMCP Dosseier]. Novato, CA: BioMarin Pharmaceutical, Inc.; 2014.
4. Elaprase [package insert]. Shire Human Genetic Therapies Inc. Cambridge MA, October 2007.
5. Naglazyme [package insert]. BioMarin Pharmaceuticals. Novato CA, June 2005.
6. Mepsevii [prescribing information].Novato, CA; Ultragenyx Pharmaceutical Inc; December 2019.

## REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
<i>Annual review</i>	<i>02/2022</i>
<i>Addition of dosing requirements and off-label restrictions</i>	<i>12/2021</i>
<i>P&amp;T Review</i>	<i>11/2020</i>