POLICY NUMBER: RX.PA.021.MPC
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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)
- Elaprase (idursulfase)
- Vimizim (elosulfase alfa)
- Mepsevii (vestronidase alfa-vjbk)

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.

Elaprase® (idursulfase) is a purified form of a lysosomal enzyme (iduronate-2-sulfatase) indicated for patients with Hunter Syndrome (Mucopolysaccharidosis II, MPS II). Elaprase® (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-vjbk) 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. Diagnosis of MPS I was confirmed by enzyme assay demonstrating a deficiency of alpha-L-iduronidase enzyme activity and/or by genetic testing.

- **Naglazyme:** Must have a confirmed diagnosis of Mucopolysaccharidosis VI (Maroteaux-Lamy syndrome). Diagnosis of MPS VI was confirmed by enzyme assay demonstrating a deficiency of N-acetylgalactosamine 4-sulfatase (arylsulfatase B) enzyme activity or by genetic testing.
- **Elaprase:** Must have a confirmed diagnosis of Hunter syndrome (Mucopolysaccharidosis type II, MPS II). Diagnosis of MPS II was confirmed by enzyme assay demonstrating a deficiency of iduronate 2-sulfatase enzyme activity or by genetic testing.
- **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). Documentation is provided that diagnosis is confirmed by enzyme assay demonstrating a deficiency of beta-glucuronidase enzyme activity or by genetic testing.

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. <u>Reauthorization Criteria:</u>

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:

<u>MPC Renewal:</u>

- Chart documentation from the prescriber that the member's condition has improved or stabilized based upon the prescriber's assessment while on therapy

<u>Renewal</u> from Previous Insurer<u>:</u>

- Members who have received prior approval (from insurer other than MPC), or have been receiving medication samples, should be considered under criterion A (Initial Authorization Criteria)

- Provider has documented clinical response of the member's condition which has stabilized or improved based upon the prescriber's assessment

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
Annual Review	*02/2026*
Annual Review	*02/2025*
Annual Review *Change in Non-MPC renewal to renewal from previous insurer*	*02/2024*

Annual review	*02/2023*
Selected Revision *Addition of MPC vs Non-MPC Renewal*	*10/2022*
Annual review	*02/2022*
Addition of dosing requirements and off-label restrictions	*12/2021*
P&T Review	*11/2020*