Clinical Policy: Elosulfase Alfa (Vimizim)

Reference Number: ERX.SPA.103
Effective Date: 10.01.16
Last Review Date: 05.20
Line of Business: Commercial, Medicaid

Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description
Elosulfase alfa (Vimizim®) is a hydrolytic lysosomal glycosaminoglycan-specific enzyme.

FDA Approved Indication(s)
Vimizim is indicated for patients with mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ that Vimizim is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Mucopolysaccharidosis IVA: Morquio A Syndrome (must meet all)**:**

1. Diagnosis of MPS IVA (Morquio A syndrome) confirmed by one of the following (a or b):
   a. Enzyme assay demonstrating a deficiency of N-acetylgalactosamine-6-sulfatase activity;
   b. DNA testing;
2. Age ≥ 5 years;
3. Dose does not exceed 2 mg per kg per week.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

A. Mucopolysaccharidosis IVA: Morquio A Syndrome (must meet all)**:**

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS IVA disease manifestation profile *(see Appendix D for examples)*;
3. If request is for a dose increase, new dose does not exceed 2 mg per kg per week.

Approval duration: 6 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
   Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off-label use policy – ERX.PA.01 or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
MPS IVA: mucopolysaccharidosis IVA

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): none reported
- Boxed warning(s): risk of life-threatening anaphylactic reactions during Vimizim infusions

Appendix D: General Information
The presenting symptoms and clinical course of MPS IVA can vary from one individual to another. Some examples, however, of improvement in MPS IVA disease as a result of Vimizim therapy may include improvement in:

- 6-minute walking test distance;
- Breathing difficulties;
- Muscle weakness;
- Vision or hearing problems;
- Height and weight;
- Hepatomegaly or splenomegaly.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MPS IVA	2 mg/kg IV once weekly	2 mg/kg/week

VI. Product Availability
Single-use vial: 5 mg/5 mL

VII. References

1. Vimizim Prescribing Information. Novato, CA: BioMarin Pharmaceutical, Inc.; December 2019. Available at http://www.vimizim.com. Accessed February 20, 2020.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.
3. Hendriksz CJ, Berger KI, Giugliani R, et al. International guidelines for the management and treatment of Morquio A syndrome. Am J Med Genet A. 2015; 167(1): 11-25.
4. Akyol MU, Alden TD, Amartino H, et al. Recommendations for the management of MPS IVA: systematic evidence- and consensus-based guidance. Orphanet J of Rare Dis 2019;14(137):1-25.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	08.16	09.16
Converted to new template. Added max dose criteria, prescriber requirement, and requirement for positive response to therapy. Decreased continued approval duration from 12 months to 6 months to allow for monitoring of therapeutic response.	06.17	08.17
2Q 2018 annual review: Removed specialist prescriber requirement; References reviewed and approved.	02.27.18	05.18

Reviews, Revisions, and Approvals	Date	P&T Approval Date
2Q 2019 annual review: no significant changes; references reviewed and updated.	02.28.19	05.19
2Q 2020 annual review: no significant changes; references reviewed and updated.	02.20.20	05.20

Important Reminder
This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.