RXADVANCE
2 Park Central Drive
Southborough, MA 01772

Clinical Policy Title:	elosulfase alfa
Policy Number:	RxA.551
Drug(s) Applied:	Vimzim®
Original Policy Date:	03/06/2020
Last Review Date:	12/07/2020
Line of Business Policy Applies to:	All lines of business

Background

Elosulfase alfa (Vimizim®) is a hydrolytic lysosomal glycosaminoglycan-specific enzyme. It is indicated for patients with mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
elosulfase alfa (Vimizim®)	MPS IVA	2 mg/kg IV once weekly	2 mg/kg/week

Dosage Forms

- Single-use vial: 5 mg/5 mL

Clinical Policy

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

I. Initial Approval Criteria

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

1. Diagnosis of Morquio A syndrome (MPS IVA) confirmed by one of the following:
   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
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

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

1. Currently receiving medication that has been authorized by RxAdvance or member has previously met initial approval criteria listed in this policy;
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
Commercial: 6 months
Medicaid: 12 months

III. Appendices

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

References

1. Vimizim® Prescribing Information. Novato, CA: BioMarin Pharmaceutical, Inc.; December 2019. Available at http://www.vimizim.com. Accessed September 21, 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.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	01/2020	03/06/2020
Policy was reviewed 1. Clinical policy title updated 2. Line of business policy applies to was updated to 'All lines of business'	09/21/2020	12/07/2020

3. Continued Therapy criteria II.A.1 was rephrased to "Currently receiving medication that has been authorized by RxAdvance..." 4. Appendix D updated to add height and weight. 5. Reference reviewed and updated.		