Clinical Policy Title:	idursulfase
Policy Number:	RxA.112
Drug(s) Applied:	Elaprase®
Original Policy Date:	02/07/2020
Last Review Date:	03/09/2021
Line of Business Policy Applies to:	All Line of Business

Background

Elaprase® is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme for patients with Hunter syndrome (mucopolysaccharidosis [MPS] II).

Elaprase® has been shown to improve walking capacity in patients 5 years and older. In patients 16 months to 5 years of age, no data are available to demonstrate improvement in disease-related symptoms or long-term clinical outcome; however, treatment with Elaprase® has reduced spleen volume similarly to that of adults and children 5 years of age and older. The safety and efficacy of Elaprase® have not been established in pediatric patients less than 16 months of age.

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
idursulfase (Elaprase®)	MPS II	0.5 mg/kg body weight IV every week	0.5 mg/kg body weight IV every week

Dosage Forms

- Injection: 6 mg/3 mL (2 mg/mL) in single-use vial

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 II: Hunter Syndrome (must meet all)**:**

1. Diagnosis of MPS II (Hunter syndrome) confirmed by one of the following (a or b):
   a. Enzyme assay demonstrating a deficiency of iduronate 2-sulfatase activity;
   b. DNA testing;
2. Age 16 months of age or older;
3. Dose does not exceed 0.5 mg per kg per week.

Approval Duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

A. Mucopolysaccharidosis II: Hunter Syndrome (must meet all)**:**

1. Member is currently receiving medication that has been authorized by RxAdvance or the member has 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 II (Hunter syndrome) manifestation profile *(see Appendix D for examples)*;
3. If request is for a dose increase, new dose does not exceed 0.5 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
FVC: forced vital capacity
MPS II: mucopolysaccharidosis II
6MWT: 6-minute walk test

APPENDIX B: Therapeutic Alternatives
Not applicable

APPENDIX C: Contraindications/Boxed Warnings

- Contraindication(s):
  - None reported.
- Boxed warning(s):
  - Risk of life-threatening anaphylactic reactions with Elaprase® infusions.

APPENDIX D: General Information

- A 10% relative improvement over baseline in the percent predicted forced vital capacity (FVC) is considered by the American Thoracic Society to be a clinically significant change and not due to week-to-week variability.
- In the clinical trials of Elaprase® in patients ≥ 5 years of age, patients treated with Elaprase® demonstrated a 35-meter mean increase relative to placebo in the 6-minute walk test (6MWT) after 53 weeks.
- The presenting symptoms and clinical course of MPS II can vary from one individual to another. Some examples, however, of improvement in MPS II disease as a result of Elaprase® therapy may include improvement in:
  - Percent predicted FVC
  - 6-minute walk test
  - Splenomegaly
  - Diarrhea
  - Joint stiffness
  - Growth deficiencies

References

1. Elaprase® Prescribing Information. Lexington, MA: Shire Human Genetic Therapies, Inc.; November 2018. Available at https://www.accessdata.fda.gov/drugsatfda_docs/label/2013/125151s0152lbl.pdf. Accessed January 18, 2021.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric

presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34. Accessed January 18, 2021.

Review/Revision History	Review/Revised Date	P&T Approval Date
Policy was established	01/2020	02/07/2020
No changes to criteria; updated references	04/29/2020	05/20/2020
Policy was reviewed Policy changes: 1. Policy title table was updated. 2. Line of business policy applies was updated to All lines of business 3. Continued therapy criteria II.A.1 was rephrased to "Currently receiving medication that has been authorized by RxAdvance…". 4. Approval duration for initial criteria updated as 6 months and " which ever is less.." removed for commercial approval duration. 5. References were reviewed and updated. 6. Dosing regimen and max dose updated to: 0.5 mg/kg body weight IV every week 7. Dosage Form updated to: Injection: 6 mg/3 mL (2 mg/mL) in single-use vial	01/18/2021	03/09/2021