Clinical Policy: Idursulfase (Elaprase)

Reference Number: PA.CP.PHAR.156
Effective Date: 01/2018
Last Review Date: 04/2025

Description
Idursulfase (Elaprase®) is a hydrolytic lysosomal glycosaminoglycan-specific enzyme.

FDA Approved Indication(s)
Elaprase is indicated for the treatment of 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.

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.

It is the policy of PA Health & Wellness that Elaprase is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Hunter Syndrome (Mucopolysaccharidosis II) (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;
3. Documentation of member's current weight (in kg);
4. Dose does not exceed 0.5 mg/kg/week.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued Approval

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

1. Currently receiving medication via PA Health & Wellness benefit or member has previously met initial approval criteria or the Continuity of Care policy (PA.PHARM.01) applies;
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. Documentation of member's current weight (in kg);
4. If request is for a dose increase, new dose does not exceed 0.5 mg/kg/week.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. Currently receiving medication via PA Health & Wellness benefit or member has previously met initial approval criteria or the Continuity of Care policy (PA.PHARM.01) applies or
2. Refer to PA.CP.PMN.53

III. Appendices/General Information

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

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MPS II	0.5 mg/kg IV every week	Based on weight

V. Product Availability

Single-use vial: 6 mg/3 mL

VI. References

1. Elaprase Prescribing Information. Lexington, MA: Shire Human Genetic Therapies, Inc.; September 2021. Available at http://www.elaprase.com. Accessed January 8, 2025.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.
3. McBride KL, Berry SA, Braverman N. Treatment of mucopolysaccharidosis type II (Hunter syndrome): a Delphi derived practice resource of the American College of Medical Genetics and Genomics (ACMG). Genetics in Medicine. 2020;22:1735-42.
4. Stapleton M, Hoshina H, Sawamoto K, et al. Critical review of current MPS guidelines and management. Molecular Genetics and Metabolism. 2019;126:238-45.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1743	Injection, idursulfase, 1 mg

Reviews, Revisions, and Approvals	Date
2Q 2018 annual review: added age restriction; HIM added; referenced reviewed and updated.	04/2018
2Q 2019 annual review: referenced reviewed and updated.	04/2019
2Q 2020 annual review: referenced reviewed and updated	04/2020
2Q 2021 annual review: no significant changes; referenced reviewed and updated.	04/2021
2Q 2022 annual review: added requirement for documentation of member's current weight for dose calculation purposes; referenced reviewed and updated.	04/2022
2Q 2023 annual review: no significant changes; references reviewed and updated.	04/2023
2Q 2024 annual review: added in continuation criteria evidenced by improvement in the individual member's MPS II (Hunter syndrome) manifestation profile; references reviewed and updated.	04/2024
2Q 2025 annual review: no significant changes; references reviewed and updated.	04/2025