Clinical Policy: Idursulfase (Elaprase)

Reference Number: ERX.SPA.100
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
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.

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 Elaprase is **medically necessary** when the following criteria are met:

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;
3. Dose does not exceed 0.5 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 II: Hunter 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 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: 12 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
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;
  - 6MWT
  - Splenomegaly;
  - Diarrhea;
  - Joint stiffness;
  - Growth deficiencies.

V. Dosage and Administration

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

VI. Product Availability

Single-use vial: 6 mg/3 mL

VII. References

1. Elaprase Prescribing Information. Lexington, MA: Shire Human Genetic Therapies, Inc.; November 2018. Available at http://www.elaprase.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.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy split from USS.SPMN.33 Lysosomal Storage Disorders and converted to new template. Added age restriction per PI. Modified approval duration to 6 months for initial and 12 months for re-auth.	08.16	09.16
Converted to new template. Added prescriber requirement. Added max dose criteria. Added requirement for positive response to therapy.	06.17	08.17
4Q17 Annual Review Removed prescriber requirement.	09.11.17	11.17
2Q 2018 annual review: No significant changes. References reviewed and updated.	02.27.18	05.18
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.