# Clinical Policy: Idursulfase (Elaprase)

Reference Number: CP.PHAR.156
Effective Date: 02.16
Last Review Date: 05.22
Line of Business: Commercial, HIM, Medicaid

Coding Implications
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.*

It is the policy of health plans affiliated with Centene Corporation® 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. Documentation of member's current weight (in kg);
4. Dose does not exceed 0.5 mg/kg per week.

**Approval duration:**
**Medicaid/HIM** – 6 months
**Commercial** – 6 months or to the member's renewal date, whichever is longer

**B. Other diagnoses/indications**

1. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

## II. Continued Therapy

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

1. Currently receiving medication via Centene benefit 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. 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 per week.

**Approval duration:**
**Medicaid/HIM** – 12 months
**Commercial** – 6 months or to the member's renewal date, whichever is longer

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

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy.
   **Approval duration: Duration of request or 6 months (whichever is less)**; or
2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

## 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 – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid, 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
  - 6-minute walk test
  - 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.; September 2021. Available at http://www.elaprase.com. Accessed February 26, 2022.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.

## 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 | P&T Approval Date |
|---|---|---|
| 2Q 2018 annual review: no significant changes; HIM added; referenced reviewed and updated. | 02.27.18 | 05.18 |
| No significant changes: added Commercial line of business per SDC | 07.31.18 | |
| 2Q 2019 annual review: no significant changes; referenced reviewed and updated. | 02.28.19 | 05.19 |

| Reviews, Revisions, and Approvals | Date | P&T Approval Date |
|---|---|---|
| 2Q 2020 annual review: no significant changes; revised HIM-Medical Benefit to HIM line of business; referenced reviewed and updated. | 02.20.20 | 05.20 |
| 2Q 2021 annual review: no significant changes; revised HIM.PHAR.21 to HIM.PA.154; referenced reviewed and updated. | 02.28.21 | 05.21 |
| 2Q 2022 annual review: no significant changes; added requirement for documentation of member's current weight for dose calculation purposes; referenced reviewed and updated. | 02.26.22 | 05.22 |

**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. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. 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. This clinical policy is not intended to

recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

**Note: For Medicaid members**, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.