



Drug Coverage Policy

Effective Date 7/15/2025

Coverage Policy Number IP0444

Policy Title Elaprase

Enzyme Replacement Therapy – Elaprase

- Elaprase® (idursulfase intravenous infusion – Shire Human Genetic Therapies)

INSTRUCTIONS FOR USE

The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Each coverage request should be reviewed on its own merits. Medical directors are expected to exercise clinical judgment where appropriate and have discretion in making individual coverage determinations. Where coverage for care or services does not depend on specific circumstances, reimbursement will only be provided if a requested service(s) is submitted in accordance with the relevant criteria outlined in the applicable Coverage Policy, including covered diagnosis and/or procedure code(s). Reimbursement is not allowed for services when billed for conditions or diagnoses that are not covered under this Coverage Policy (see "Coding Information" below). When billing, providers must use the most appropriate codes as of the effective date of the submission. Claims submitted for services that are not accompanied by covered code(s) under the applicable Coverage Policy will be denied as not covered. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

OVERVIEW

Elaprase, human iduronate-2-sulfatase (idursulfase), is indicated for **Hunter syndrome (Mucopolysaccharidosis type II [MPS II])**.¹

Disease Overview

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MPS II or Hunter syndrome, is a rare, X-linked lysosomal storage disorder characterized by a deficiency of iduronate-2-sulfatase leading to the accumulation of glycosaminoglycans dermatan sulfate and heparin sulfate.^{2,3} Males are almost exclusively affected, although there have been a few case reports of females with Hunter syndrome.^{3,4} The onset, progression, and severity of MPS II is variable.²⁻⁴ Most of the patients with MPS II have a severe form with neurologic involvement leading to cognitive impairment and neurologic regression.^{3,4} Other manifestations of Hunter syndrome include coarse facial features, hepatosplenomegaly, cardiac and respiratory disease, short stature, and stiff joints and contractures.^{2,3} The definitive diagnosis of MPS II is established by demonstrating deficient iduronate-2-sulfatase activity in leukocytes, fibroblasts, serum, or plasma; or mutations in the iduronate-2-sulfatase gene.^{2,5} Definitive treatment of MPS II consists of enzyme replacement therapy with Elaprase.^{2-4,6} Hematopoietic stem cell transplantation has not demonstrated clear neurological benefit to date and is not recommended for MPS II due to the high rate of morbidity and mortality associated with this therapy.^{2,4}

Coverage Policy

POLICY STATEMENT

Prior Authorization is required for benefit coverage of Elaprase. Approval is recommended for those who meet the Criteria and Dosing for the listed indication. Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist). All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Elaprase as well as the monitoring required for adverse events and long-term efficacy, approval requires Elaprase to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Elaprase is considered medically necessary when the following are met:

FDA-Approved Indication

- 1. Mucopolysaccharidosis Type II (Hunter Syndrome).** Approve for 1 year if the patient meets BOTH of the following (A and B):
 - A)** The diagnosis is established by ONE of the following (i or ii):
 - i. Patient has a laboratory test demonstrating deficient iduronate-2-sulfatase activity in leukocytes, fibroblasts, serum, or plasma; OR
 - ii. Patient has a molecular genetic test demonstrating an iduronate-2-sulfatase gene variant; AND
 - B)** Elaprase is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Dosing. Each dose must not exceed 0.5 mg/kg administered intravenously no more frequently than once a week.

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Receipt of sample product does not satisfy any criteria requirements for coverage.

Conditions Not Covered

Elaprase for any other use is considered not medically necessary. Criteria will be updated as new published data are available.

Coding Information

Note: 1) This list of codes may not be all-inclusive.
2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

Considered Medically Necessary when criteria in the applicable policy statements listed above are met:

HCPSC Codes	Description
J1743	Injection, idursulfase, 1 mg

References

1. Elaprase® intravenous infusion [prescribing information]. Cambridge, MA: Takeda; February 2025.
2. Scarpa M, Almassy Z, Beck M, et al. Mucopolysaccharidosis type II: European recommendations for the diagnosis and multidisciplinary management of a rare disease. *Orphanet J Rare Dis.* 2011;6:72.
3. Muenzer J, Beck M, Eng CM, et al. Multidisciplinary management of Hunter syndrome. *Pediatrics.* 2009;124:e1228-e1239.
4. Giugliani R, Federhen A, Munoz Rojas MV, et al. Mucopolysaccharidosis I, II, and VI: Brief review and guidelines for treatment. *Genet Mol Biol.* 2010;33:589-604.
5. D'Avanzo F, Rigon L, Zanetti A, Tomanin R. Mucopolysaccharidosis type II: One hundred years of research, diagnosis, and treatment. *Int J Mol Sci.* 2020;21:E1258.
6. McBride KL, Berry SA, Braverman N; ACMG Therapeutics Committee. Treatment of mucopolysaccharidosis type II (Hunter syndrome): a Delphi derived practice resource of the American College of Medical Genetics and Genomics (ACMG). *Genet Med.* 2020 Nov;22(11):1735-1742.

Revision Details

Type of Revision	Summary of Changes	Date
Annual Revision	Added dosing	8/1/2024
Annual Revision	No criteria changes	7/15/2025

The policy effective date is in force until updated or retired

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