Elaprase® (idursulfase)
(Intravenous)

Effective Date: 03/01/2020
Review Date: 01/28/2021, 02/11/2021, 1/27/2022, 4/14/2022, 01/19/2023, 12/7/2023, 01/10/2024, 5/21/2025

I. Length of Authorization

Coverage will be provided for 12 months and may be renewed.

II. Dosing Limits

A. Max Units (per dose and over time) [HCPCS Unit]:

- 240 billable units every 28 days

III. Summary of Evidence

Elaprase (idursulfase) is approved for the treatment of Hunter syndrome (Mucopolysacchardisosis II, MPS II), an X-linked lysosomal storage disorder due to iduronate-2-sulfatase deficiency. Its approval is based on 52-week randomized, placebo-controlled trial in 96 male patients aged 5-31 years. Patients treated with Elaprase 0.5 mg/kg weekly demonstrated significant improvement in a composite efficacy endpoint based on 6-minute walk test (mean increase of 35 meters vs. placebo) and percent predicted forced vital capacity (FVC), although the FVC improvement alone was not statistically significant. Patients demonstrated sustained improvements in walking distance and reductions in urinary GAG levels and spleen volume. The most common adverse reactions were hypersensitivity reactions such as rash, urticaria, pyrexia, and headache. Anaphylactic reactions were reported in 15% of patients, occurring during or after infusions. Immune response, including development of anti-idursuflase antibodies and neutralizing antibodies, was seen in 51% of patients ≥ 5 years old and in 68% of those < 7 years older. Elaprase is administered as a weekly intravenous infusion. Due to the risk of severe infusion reactions and antibody development, patients must be monitored closely and premedicated if necessary.

IV. Initial Approval Criteria[1,4,5,7,9,10]

Coverage is provided in the following conditions:

Medicare members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

- Patient is at least 16 months of age; **AND**
- Therapy is being used to treat non-central nervous system manifestations of the disease and patient does not have severe, irreversible cognitive impairment; AND
- Documented baseline values for one or more of the following have been obtained:
    - Patients 5 years or greater: 6-minute walk test (6-MWT), percent predicted forced vital capacity (FVC), joint range of motion, left ventricular hypertrophy, growth, quality of life (CHAQ/HAQ/MPS HAQ), and/or urinary glycosaminoglycan (uGAG); **OR**

- Patients 16 months to less than < 5 years of age: spleen volume, liver volume, FVC, 6-minute walk test, and/or urinary glycosaminoglycan (uGAG); **AND**

Hunter syndrome (Mucopolysaccharidosis II; MPS II) † Φ

- Patient has definitive diagnosis of MPS II as confirmed by one of the following:
  - Deficient or absent iduronate 2-sulfatase (I2S) enzyme activity in white cells, fibroblasts, or plasma in the presence of normal activity of at least one other sulfatase; **OR**
  - Detection of pathogenic mutations in the *IDS* gene by molecular genetic testing

† FDA Approved Indication(s); **Φ** Orphan Drug

V. Renewal Criteria[1,4,5,7,9,10]

Coverage may be renewed based on the following criteria:

- Patient continues to meet indication-specific relevant criteria identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: severe hypersensitivity reactions including anaphylaxis, antibody development and serious adverse reactions in Hunter Syndrome patients with severe genetic mutations, acute respiratory complications, acute cardiorespiratory failure, etc.; **AND**
- Patient has demonstrated a beneficial response to therapy compared to pretreatment age- appropriate baseline values in one or more of the following:
  - Patients 5 years or greater: stabilization or improvement in percent predicted FVC and/or 6-minute walk test, increased joint range of motion, decreased left ventricular hypertrophy, improved growth, improved quality of life (clinically meaningful change in the CHAQ/HAQ/MPS HAQ disability index), and/or uGAG levels; **OR**
  - Patients 16 months to less than < 5 years of age: reductions in spleen volume and/or liver volume or stabilization/improvement in FVC and/or 6-MWT, or uGAG levels

VI. Dosage/Administration[1,9,10]

Indication	Dose
Hunter Syndrome; MPS II	0.5 mg/kg of body weight administered once weekly as an intravenous infusion

VII. Billing Code/Availability Information

HCPCS Code:

- J1743 – Injection, idursulfase, 1 mg; 1 mg = 1 billable unit

NDC:

- Elaprase 6 mg/3 mL single-use vial for injection: 54092-0700-xx

VIII. References

1. Elaprase [package insert]. Lexington, MA; Shire Human Genetic Therapies, Inc; October 2022. Accessed May 2025.
2. Wraith JE, Scarpa M, Beck M, et al. Mucopolysaccharidosis type II (Hunter syndrome): a clinical review and recommendations for treatment in the era of enzyme replacement therapy. Eur J Pediatr. 2008 Mar;167(3):267-77. Epub 2007 Nov 23.
3. Scarpa M, Almássy 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 Nov 7;6:72. doi: 10.1186/1750-1172-6-72.
4. Muenzer J, Bodamer O, Burton B, et al. The role of enzyme replacement therapy in severe Hunter syndrome-an expert panel consensus. Eur J Pediatr. 2012 Jan;171(1):181-8.
5. Scarpa M. Mucopolysaccharidosis Type II. GeneReviews®. www.ncbi.nlm.nih.gov/books/NBK1274/. Initial Posting: November 6, 2007; Last Update: October 4, 2018. Accessed on December 26, 2019.
6. Burrow T, Leslie ND. Review of the use of idursulfase in the treatment of mucopolysaccharidosis II. Biologics. 2008 Jun; 2(2): 311–320.
7. Giugliani R, Villareal MLS, Valdez CAA, et al. Guidelines for diagnosis and treatment of Hunter Syndrome for clinicians in Latin America. Genet Mol Biol. 2014 Jun; 37(2): 315–329.
8. Burton BK, Giugliani R. Diagnosing Hunter syndrome in pediatric practice: practical considerations and common pitfalls. Eur J Pediatr 2012; 171:631.
9. Muenzer J, Wraith J, Beck M, *et al.* A phase II/III clinical study of enzyme replacement therapy with idursulfase in mucopolysaccharidosis II (Hunter syndrome). *Genet Med* **8,** 465–473 (2006) doi:10.1097/01.gim.0000232477.37660.fb
10. Muenzer J, Beck M, Eng CM, et al. Long-term, open-labeled extension study of idursulfase in the treatment of Hunter syndrome. Genet Med. 2011 Feb;13(2):95-101. doi: 10.1097/GIM.0b013e3181fea459.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E76.1	Mucopolysaccharidosis, type II

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Determinations (LCDs), and Local Coverage Articles (LCAs) may exist and compliance with these policies is required where applicable. They can be found at: http://www.cms.gov/medicare-coverage-database/search/advanced-search.aspx. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC

Policy Rationale:

Elaprase was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Elaprase according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For Medicare members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.