



Elaprase® (idursulfase) (Intravenous)

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I. Length of Authorization

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

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC unit]:

- Elaprase 6 mg vial: 10 vials per 7 days

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

- 60 billable units every 7 days

III. Initial Approval Criteria^{1,4,5,7,9,10}

Coverage is provided in the following conditions:

Universal Criteria

- Patient is at least 16 months old; **AND**

Hunter syndrome (Mucopolysaccharidosis II; MPS II) †

- Patient has absence of severe cognitive impairment; **AND**
- Diagnosis has been 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; **AND**
- Documented baseline value for urinary glycosaminoglycan (uGAG); **AND**
- Documented baseline values for one or more of the following:

- Patients 5 years or greater: 6-minute walk test (6-MWT), percent predicted forced vital capacity (FVC), joint range of motion, left ventricular hypertrophy, quality of life (CHAQ/HAQ/MPS HAQ); **OR**
 - Patients < 5 years: spleen volume, liver volume, FVC, and/or 6-minute walk test
- † FDA Approved Indication(s)

IV. Renewal Criteria^{1,4,5,7,9,10}

Authorizations can be renewed based on the following criteria:

- Patient continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. 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 does not have progressive/irreversible severe cognitive impairment; **AND**
- Patient has a documented reduction in uGAG levels; **AND**
- Patient has demonstrated a beneficial response to therapy compared to pretreatment baseline 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); **OR**
 - Patients < 5 years: spleen volume, and/or liver volume or stabilization/improvement in FVC and/or 6-MWT

V. 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

VI. 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

VII. References

1. Elaprase [package insert]. Lexington, MA; Shire Human Genetic Therapies, Inc; November 2018. Accessed December 2019.
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 Articles 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/Article): 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 Government Benefit Administrators, 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