

Kanuma® (sebelipase alfa) (Intravenous)

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I. Length of Authorization

Initial coverage will be provided for 6 months and may be renewed annually thereafter.

II. Dosing Limits

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

- 340 billable units every 14 days

III. Initial Approval Criteria ¹

Coverage is provided in the following conditions:

- Patient is at least 1 month of age; **AND**
- Documented baseline values for one or more of the following have been obtained:
 - Weight-for-age z-scores for patients exhibiting growth failure
 - Low-density lipoprotein cholesterol (LDL-c)
 - High-density lipoprotein cholesterol (HDL-c)
 - Non-HDL-c
 - Triglycerides
 - Aspartate aminotransferase (AST) and/or alanine aminotransferase (ALT)

Lysosomal Acid Lipase (LAL) Deficiency † Φ ^{1,6-8}

- Diagnosis has been confirmed by either biallelic pathogenic variants in *LIPA* or deficient LAL enzyme activity in peripheral blood leukocytes, fibroblasts, or dried blood spots

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria ^{1,6,7}

Coverage can be renewed based upon the following criteria:

- Patient continues to meet 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: hypersensitivity reactions including anaphylaxis, etc.; **AND**
 - Treatment has resulted in clinical benefit as evidenced in one or more of the following:
 - Improvement in weight-for-age z-scores for patients exhibiting growth failure
 - Improvement in LDL-c
 - Improvement in HDL-c
 - Improvement in non-HDL-c
 - Improvement in triglycerides
 - Improvement of AST and/or ALT; **OR**
- Dose escalation** in pediatric and adult patients with a suboptimal clinical response to the 1 mg/kg dose defined by at least one of the following:
 - Poor growth
 - Deteriorating biochemical markers (e.g., ALT, AST), and/or parameters of lipid metabolism (e.g., LDL-c, triglycerides); **OR**
- Dose escalation** for infants with rapidly progressive disease presenting within the first 6 months of life who have a suboptimal clinical response to the 1 mg/kg dose or 3 mg/kg dose defined by at least one of the following:
 - Poor growth
 - Deteriorating biochemical markers (e.g., ALT, AST)
 - Persistent or worsening organomegaly

V. Dosage/Administration ¹

Indication	Dose
LAL Deficiency	<u>Pediatric & Adult patients:</u>
	<ul style="list-style-type: none"> • 1 mg/kg administered once every other week as an intravenous infusion • May increase dose to 3 mg/kg once every other week for patients who do not achieve an optimal clinical response to the 1 mg/kg dose**
	<u>Infants with rapidly progressive disease presenting within the first 6 months of life:</u>
	<ul style="list-style-type: none"> • 1 mg/kg administered once weekly as an intravenous infusion

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| <ul style="list-style-type: none"> • May increase dose to 3 mg/kg once weekly for patients who do not achieve an optimal clinical response** • May further increase dose to 5 mg/kg once weekly for patients who do not achieve an optimal clinical response to the 3 mg/kg dose** <p><i>**Refer to dose escalation criteria in section IV</i></p> |
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VI. Billing Code/Availability Information

HCPCS Code:

- J2840 – Injection, sebelipase alfa, 1 mg; 1 billable unit = 1 mg

NDC:

- Kanuma 20 mg/10 mL single-dose vial: 25682-0007-xx

VII. References

1. Kanuma [package insert]. Boston, MA; Alexion Pharmaceuticals, Inc; July 2024. Accessed March 2025.
2. Porto AF. Lysosomal acid lipase deficiency: diagnosis and treatment of Wolman and Cholesteryl Ester Storage Diseases. *Pediatr Endocrinol Rev.* 2014 Sep;12 Suppl 1:125-32.
3. Zhang B, Porto AF. Cholesteryl ester storage disease: protean presentations of lysosomal acid lipase deficiency. *Pediatr Gastroenterol Nutr.* 2013;56(6):682.
4. Reiner Z, Guardamagna O, Nair D, et al. Lysosomal acid lipase deficiency--an under-recognized cause of dyslipidaemia and liver dysfunction. *Atherosclerosis.* 2014 Jul;235(1):21-30. doi: 10.1016/j.atherosclerosis.2014.04.003.
5. Hamilton J, Jones I, Srivastava R. A new method for the measurement of lysosomal acid lipase in dried blood spots using the inhibitor Lalistat 2. *Clin Chim Acta.* 2012 Aug 16;413(15-16):1207-10. doi: 10.1016/j.cca.2012.03.019.
6. Burton BK, Balwani M, Feillet F, et al. A Phase 3 Trial of Sebelipase Alfa in Lysosomal Acid Lipase Deficiency. 2015 Sep 10;373(11):1010-20. doi: 10.1056/NEJMoa1501365.
7. Jones SA, Rojas-Caro S, Quinn AG, et al. Survival in infants treated with sebelipase Alfa for lysosomal acid lipase deficiency: an open-label, multicenter, dose-escalation study. *Orphanet J Rare Dis.* 2017 Feb 8;12(1):25. doi: 10.1186/s13023-017-0587-3.
8. Hoffman EP, Barr ML, Giovanni MA, et al. Lysosomal Acid Lipase Deficiency. 2015 July 30 [Updated 2016 September 1]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. *GeneReviews*® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2023. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK305870/>.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E75.5	Other lipid storage disorders

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

The preceding information is intended for non-Medicare coverage determinations. 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 Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: <https://www.cms.gov/medicare-coverage-database/search.aspx>. Additional indications, including any preceding information, may be applied 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
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
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