

Kanuma™ (sebelipase alfa)

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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

A. Quantity Limit (max daily dose) [Pharmacy Benefit]:

- Kanuma 20 mg/10 mL single-use vials: 68 vials per 28 day supply

B. Max Units (per dose and over time) [Medical Benefit]:

- 340 billable units once weekly

III. Initial Approval Criteria

Coverage is provided in the following conditions:

Lysosomal Acid Lipase (LAL) deficiency †

- 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; **AND**
- Patient is at least 1 month old

† FDA Approved Indication(s)

IV. Renewal Criteria

Coverage can be renewed based upon the following criteria:

- Patient continues to meet the criteria identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: hypersensitivity reactions (anaphylaxis, abdominal pain, fever, chills, pruritus, rash, vomiting), etc. ; **AND**
- Treatment has resulted in clinical benefit; for example:
 - Improvement in weight-for-age z-scores for patients exhibiting growth failure
 - Improvement in LDL

- Improvement in HDL
- Improvement in triglycerides
- Improvement of AST or ALT

V. Dosage/Administration

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 IV infusion
	<u>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 IV infusion • May increase to 3 mg/kg once weekly for patients who do not achieve an optimal clinical response

VI. Billing Code/Availability Information

Jcode:

J2840 - Injection, sebelipase alfa, 1 mg

NDC(s):

Kanuma 20 mg/10 mL single-use vials: 25682-0007-01

VII. References

1. Kanuma [package insert]. Cheshire, CT; Alexion Pharmaceuticals, Inc; December 2015. Accessed March 2018.
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. 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.
4. 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.
5. 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.

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)

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) and Local Coverage Determinations (LCDs) 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):

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 Corporation (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 Corporation (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