Effective Date: 12/2017
Reviewed: 12/2017, 11/2018, 12/2019, 09/2020, 04/2021, 3/2022, 01/2023, 12/2023, 01/2024 Pharmacy Benefit Scope: Medicaid Medical Benefit Scope: Commercial, Medicare-Medicaid Plan (MMP)

Kanuma™ (sebelipase alfa)
(Intravenous)

I. Length of Authorization

Initial & Renewal coverage will be provided for 6 months.

II. Dosing Limits

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

- Kanuma 20 mg/10 mL single-dose vials: 112 vials per 28 day supply

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

- 560 billable units once weekly

III. Summary of Evidence

Kanuma is a hydrolytic lysosomal cholesteryl ester and triacylglycerol-specific enzyme indicated for the treatment of patients with a diagnosis of Lysosomal Acid Lipase (LAL) deficiency. Clinical trials evaluating the efficacy and safety of Kanuma have demonstrated significant improvements in key clinical endpoints, including reduction of hepatic fat content, normalization of liver enzymes, and improvement in lipid profiles. Kanuma further has been shown to effectively address the underlying enzyme deficiency in patients with LAL deficiency, thereby reducing the risk of hepatic and extrahepatic manifestations associated with the disease.

IV. Initial Approval Criteria [1-6]

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

Coverage is provided in the following conditions:

- Patient is at least 1 month of age; AND
- Prescribing physician is a specialist in genetics and metabolism; AND
- Weight, baseline liver function and baseline lipid panel is provided;

Lysosomal Acid Lipase (LAL) Deficiency † Φ

- 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); **Φ** Orphan Drug

V. Renewal Criteria [1-6]

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
        - Improvement in HDL
        - Improvement in triglycerides
        - Improvement of AST 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., alanine aminotransferase (ALT), aspartate aminotransferase (AST)], and/or parameters of lipid metabolism [e.g., low-density lipoprotein cholesterol (LDL-c), triglycerides (TG)]; **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., alanine aminotransferase (ALT), aspartate aminotransferase (AST)]
        - Persistent or worsening organomegaly

VI. Dosage/Administration[1]

Indication	Dose
LAL deficiency	<u>Pediatric & Adult patients:</u> • 1 mg/kg administered once every other week as an IV 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

	• Infants with rapidly progressive disease presenting within the first 6 months of life:1 mg/kg administered once weekly as an IV infusion • 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

VII. Billing Code/Availability Information

HCPCS Code:

J2840 - Injection, sebelipase alfa, 1 mg: 1 billable unit = 1 mg

NDC(s):

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

VIII. References

1. Kanuma [package insert]. Boston, MA; Alexion Pharmaceuticals, Inc; July 2023. Accessed October2023.
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, Port 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.

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), 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:

Kanuma 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 Kanuma 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 INTEGRITY (Medicare-Medicaid Plan) 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.