Effective Date: 12/2017
Reviewed: 12/2017, 11/2018, 12/2019, 09/2020, 04/2021, 3/2022, 01/2023, 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. Initial Approval Criteria [1-6]

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

IV. 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

V. Dosage/Administration[1]

Indication	Dose
LAL deficiency	Pediatric & Adult patients: • 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

VI. 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

VII. 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