

Policy Title:	Kanuma (sebelipase alfa) (Intravenous)		
		Department:	PHA
Effective Date:	01/01/2020		
Review Date:	12/13/2019		
Revision Date:	12/13/2019		

Purpose: To support safe, effective and appropriate use of Kanuma (sebelipase alfa).

Scope: Medicaid, Exchange, Medicare-Medicaid Plan (MMP)

Policy Statement:

Kanuma (sebelipase alfa) is covered under the Medical Benefit when used within the following guidelines. Use outside of these guidelines may result in non-payment unless approved under an exception process.

Procedure:

Coverage of Kanuma (sebelipase alfa) will be reviewed prospectively via the prior authorization process based on criteria below.

Initial Criteria:

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
- Lab values obtained for LDL, HDL, triglycerides, and AST/ALT
- Documentation of weight

Continuation of Therapy Criteria:

- Patient continues to meet initial criteria; 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

Coverage durations:

- Initial coverage: 6 months
- Continuation of therapy coverage: 6 months

*** Requests will also be reviewed to National Coverage Determination (NCD) and Local Coverage Determinations (LCDs) if applicable. ***

Dosage/Administration:

Indication	Dose	Maximum dose (1 billable unit = 1 mg)
LAL deficiency	<p><u>Pediatric & Adult patients:</u></p> <ul style="list-style-type: none"> • 1 mg/kg administered once every other week as an IV infusion <p><u>Rapidly progressive disease presenting within the first 6 months of life:</u></p> <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 	340 billable units once weekly

Investigational use: All therapies are considered investigational when used at a dose or for a condition other than those that are recognized as medically accepted indications as defined in any one of the following standard reference compendia: American Hospital Formulary Service Drug information (AHFS-DI), Thomson Micromedex DrugDex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs, or Peer-reviewed published medical literature indicating that sufficient evidence exists to support use. Neighborhood does not provide coverage for drugs when used for investigational purposes.

Applicable Codes:

Below is a list of billing codes applicable for covered treatment options. The below tables are provided for reference purposes and may not be all-inclusive. Requests received with codes from tables below do not guarantee coverage. Requests must meet all criteria provided in the procedure section.

The following HCPCS/CPT code is:

HCPCS/CPT Code	Description
J2840	Injection, sebelipase alfa, 1mg

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 underrecognized 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.