

<b>Policy Title:</b>	Kanuma (sebelipase alfa) (Intravenous)		
<b>Policy Number:</b>	<i>To be determined</i>	<b>Department:</b>	PHA
<b>Effective Date:</b>	01/01/2020		
<b>Review Date:</b>	09/25/2019, 1/15/2020		
<b>Revision Date:</b>	09/25/2019, 1/15/2020		

**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 enzyme assay demonstrating a deficiency of lysosomal acid lipase enzyme activity or genetic testing revealing mutation in the lipase A, lysosomal acid type (LIPA) gene; AND
- Patient is at least 1 month old; AND
- Prescribing physician is a specialist in genetics and metabolism; AND
- Weight, baseline liver function and baseline lipid panel is provided;
- MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

***Continuation of Therapy Criteria:***

- Patient continues to meet initial criteria; AND
- Patient is tolerating treatment; AND
- Recent weight, liver function and lipid panel after treatment initiation is provided; AND

- Documented clinical benefit with therapy (e.g. reduction in LDL, triglycerides, AST or ALT, increase in HDL, improvement in weight-for-age z-scores, reduction in liver fat content)

#### 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	<u>Pediatric &amp; Adult patients:</u> <ul style="list-style-type: none"> <li>• 1 mg/kg administered once every other week as an IV infusion</li> </ul> <u>Rapidly progressive disease presenting within the first 6 months of life:</u> <ul style="list-style-type: none"> <li>• 1 mg/kg administered once weekly as an IV infusion</li> <li>• May increase to 3 mg/kg once weekly for patients who do not achieve an optimal clinical response</li> </ul>	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:

HCP/CS/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.