

Clinical Policy: Sebelipase Alfa (Kanuma)

Reference Number: LA.PHAR.159

Effective Date:

Last Review Date: 08.22

Line of Business: Medicaid

[Coding Implications](#)

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

Sebelipase alfa (Kanuma®) is a hydrolytic lysosomal cholesteryl ester and triacylglycerol-specific enzyme.

FDA Approved Indication(s)

Kanuma is indicated for the treatment of patients with a diagnosis of lysosomal acid lipase (LAL) deficiency.

Policy/Criteria

Prior Authorization is required. Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of Louisiana Healthcare Connections that Kanuma is medically necessary when the following criteria are met:

I. Initial Approval Criteria

A. Lysosomal Acid Lipase Deficiency (must meet all):

1. Diagnosis of LAL deficiency confirmed by one of the following (a or b):
 - a. Enzyme assay demonstrating a deficiency of LAL activity;
 - b. Lipase A - lysosomal acid type (LIPA) gene mutation;
2. Age ≥ 1 month;
3. Documentation of member's current weight (in kg);
4. Request meets one of the following (a or b):
 - a. Dose does not exceed 3 mg/kg every other week;
 - b. For members with rapidly progressive disease presenting within the first 6 months of life: Dose does not exceed any of the following (i or ii):
 - i. 3 mg/kg per week;
 - ii. 5 mg/kg per week, upon documentation of suboptimal clinical response to 3 mg/kg per week.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): LA.PMN.53 for Medicaid.

II. Continued Therapy

A. Lysosomal Acid Lipase Deficiency (must meet all):

1. Currently receiving medication via Louisiana Healthcare Connections benefit or member has previously met initial approval criteria;
2. Member is responding positively to therapy as evidenced by documentation of clinical response which may include, but is not limited to:
 - a. For members with rapidly progressive disease presenting within first 6 months of life: continued survival;
 - b. For all other members: decrease in low-density lipoprotein cholesterol (LDL-c), non-high-density lipoprotein cholesterol (non-HDL-c), or triglycerides; increase in HDL-c; normalization of alanine aminotransferase (ALT) or aspartate aminotransferase (AST); reduction in hepatic fat content, steatosis, or liver volume;
3. Documentation of member's current weight (in kg);
4. If request is for a dose increase, new dose does not exceed any of the following (a or b):
 - a. 3 mg/kg every other week;
 - b. For members with rapidly progressive disease presenting within the first 6 months of life: Dose does not exceed any of the following (i or ii):
 - i. 3 mg/kg per week;
 - ii. 5 mg/kg per week, upon documentation of suboptimal clinical response to 3 mg/kg per week .

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via Louisiana Healthcare Connections benefit and documentation supports positive response to therapy.
Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): LA.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – LA.PMN.53 for Medicaid, or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

ALT: alanine aminotransferase

AST: aspartate aminotransferase

FDA: Food and Drug Administration

HDL-c: non-high-density lipoprotein cholesterol

LAL: lysosomal acid lipase

LDL-c: low-density lipoprotein cholesterol

LIPA: lipase A – lysosomal acid type

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Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

None reported

Appendix D: Measures of Therapeutic Response

- LAL normally causes the breakdown of lipid particles, including LDL-c. A lack of LAL results in accumulation of cholesteryl esters and triglycerides. Therefore, LDL-c, non-HDL-c, triglycerides, and HDL-c are clinical parameters that can indicate therapeutic response to Kanuma. In clinical trials, there were initial increases in LDL-c and triglycerides within the first 2-4 weeks of treatment; however, this was followed by a decrease to below pre-treatment values within 8 weeks of treatment.
- In addition, the lipid accumulation seen in LAL deficiency can occur in multiple organs, including the liver. This results in increased liver fat content and progression of liver disease, including fibrosis and cirrhosis. In clinical trials, patients receiving Kanuma had normalization of ALT and AST levels, reduction in hepatic fat content and steatosis (defined as the absolute decrease of $\geq 5\%$ from baseline in assessment of hepatic fat content)* and decrease in baseline liver volume* when compared to patients receiving placebo. As such, improvement in these areas may also indicate positive response to Kanuma.

*Not statistically significant

V. Dosage and Administration

<u>Indication</u>	<u>Dosing Regimen</u>	<u>Maximum Dose</u>
<u>LAL deficiency: rapidly progressive disease presenting within first 6 months of life</u>	<u>1 mg/kg IV once weekly</u> <u>For patients with a suboptimal clinical response, increase the dosage to 3 mg/kg once weekly.</u> <u>For patients with continued suboptimal clinical response, further increase the dosage to 5 mg/kg once weekly.</u>	<u>5 mg/kg/week</u>
<u>LAL deficiency</u>	<u>1 mg/kg IV every other week</u> <u>For patients with a suboptimal clinical response, increase the dosage to 3 mg/kg once every other week.</u>	<u>3 mg/kg every other week</u>

VI. Product Availability

Single-use vial: 20 mg/10 mL

VII. References

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1. Kanuma Prescribing Information. Cheshire, CT: Alexion Pharmaceuticals, Inc.; Cambridge, MA: Genzyme Corporation; November 2021. Available at <http://www.kanuma.com/>. Accessed February 26, 2022.
2. Zhang B, Porto AF. Cholesteryl ester storage disease: protean presentations of lysosomal acid lipase deficiency. J Pediatr Gastroenterol Nutr. 2013;56(6):682.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

<u>HCPCS Codes</u>	<u>Description</u>
J2840	Injection, sebelipase alfa, 1 mg

<u>Reviews, Revisions, and Approvals</u>	<u>Date</u>	<u>LDH Approval Date</u>
Converted corporate to local policy.	09.22	

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. LHCC makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved.

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This clinical policy is effective as of the date determined by LHCC. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable

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legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. LHCC retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

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