Clinical Policy: Sebelipase Alfa (Kanuma)

Reference Number: LA.PHAR.159
Effective Date: 11.04.23
Last Review Date: 06.10.24
Line of Business: Medicaid

Coding Implications
Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

****Please note: This policy is for medical benefit****

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

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.*
      **Suboptimal clinical response is defined as any of the following: poor growth, deteriorating biochemical markers, or persistent or worsening organomegaly.*

Approval duration: 6 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to LA.PMN.255
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: 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.*
      **Suboptimal clinical response is defined as any of the following: poor growth, deteriorating biochemical markers, or persistent or worsening organomegaly.*

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to LA.PMN.255
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy LA.PMN.53

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

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

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 ≥ 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

Indication	Dosing Regimen	Maximum Dose
LAL deficiency: rapidly progressive disease presenting within first 6 months of life	1 mg/kg IV once weekly For patients with a suboptimal clinical response, increase the dosage to 3 mg/kg once weekly. For patients with continued suboptimal clinical response, further increase the dosage to 5 mg/kg once weekly.* **Suboptimal clinical response is defined as any of the following: poor growth, deteriorating biochemical markers, or persistent or worsening organomegaly.*	5 mg/kg/week
LAL deficiency	1 mg/kg IV every other week	3 mg/kg every other week

Indication	Dosing Regimen	Maximum Dose
	For patients with a suboptimal clinical response, increase the dosage to 3 mg/kg once every other week.** ***Suboptimal clinical response is defined as any 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)].*	

VI. Product Availability

Single-use vial: 20 mg/10 mL

VII. References

1. Kanuma Prescribing Information. Cheshire, CT: Alexion Pharmaceuticals, Inc.; Cambridge, MA: Genzyme Corporation; November 2021. Available at http://www.kanuma.com/. Accessed January 9, 2024.
2. Zhang B, Porto AF. Cholesteryl ester storage disease: protean presentations of lysosomal acid lipase deficiency. J Pediatr Gastroenterol Nutr. 2013;56(6):682.
3. Kohli R, Ratziu V, Fiel MI, et al. Initial assessment and ongoing monitoring of lysosomal acid lipase deficiency in children and adults: consensus recommendations from an international collaborative working group. Molecular Genetics and Metabolism. 2020;129:59-66.

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.

HCPCS Codes	Description
J2840	Injection, sebelipase alfa, 1 mg

Reviews, Revisions, and Approvals	Date	LDH Approval Date
Converted corporate to local policy.	09.22	09.15.22
Template changes applied to other diagnoses/indications and continued therapy section. No significant changes; added definition of "suboptimal clinical response" for determining the need for further dose increases; references reviewed and updated.	06.02.23	10.05.23
Annual review: no significant changes; references reviewed and updated.	06.10.24	

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.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable LHCC administrative policies and procedures.

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This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

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