Clinical Policy: Sebelipase Alfa (Kanuma)

Reference Number: ERX.SPA.107
Effective Date: 10.01.16
Last Review Date: 05.20
Line of Business: Commercial, Medicaid

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

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ 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. Dose does not exceed 1 mg per kg every other week (*1 mg per kg per week for members with rapidly progressive disease presenting within first 6 months of life; may be increased to 3 mg per kg per week upon documentation of suboptimal clinical response to 1 mg per kg per week*).

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

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

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions 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: 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. If request is for a dose increase, new dose does not exceed 1 mg per kg every other week (*1 mg per kg per* week *for members with rapidly progressive disease presenting within first 6*

months of life; may be increased to 3 mg per kg per week upon documentation of suboptimal clinical response to 1 mg per kg per week).
Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

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 policy – ERX.PA.01 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

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	3 mg/kg/week
LAL deficiency	1 mg/kg IV every other week	1 mg/kg every other week

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; December 2015. Available at http://www.kanuma.com/. Accessed February 21, 2020.
2. Zhang B, Porto AF. Cholesteryl ester storage disease: protean presentations of lysosomal acid lipase deficiency. J Pediatr Gastroenterol Nutr. 2013; 56(6):682.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	08.16	09.16
Converted to new template. Added prescriber requirement. Added max dose criteria. Added requirement for positive response to therapy.	06.17	08.17
4Q17 Annual Review Removed prescriber requirement.	09.11.17	11.17
2Q 2018 annual review: No significant changes. References reviewed and updated.	02.26.18	05.18
2Q 2019 annual review: no significant changes; references reviewed and updated.	02.28.19	05.19
2Q 2020 annual review: no significant changes; references reviewed and updated.	02.21.20	05.20

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.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. 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.