Medical Drug Clinical Criteria

Subject:	Kanuma (sebelipase alfa)		
Document #:	CC-0037	**Publish Date**:	~~05/06/2024~~04/24/2025
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Overview

This document addresses the use of Kanuma (sebelipase alfa), a hydrolytic lysosomal cholesteryl ester and triacylglycerol-specific enzyme administered intravenously for the treatment of the rare disease lysosomal acid lipase deficiency (LAL-D), also known as Wolman disease (WD) and cholesteryl ester storage disease (CESD).

LAL-D is a rare autosomal recessive metabolic disease that ranges in severity and age of onset. Infants with a rapidly progressive form of LAL-D, also known as WD, rarely survive beyond 1 year of age and are believed to have complete loss of lysosomal acid lipase. The later-onset forms, associated with partial enzyme loss, are collectively known as CESD which present in childhood with lipid abnormalities, elevated liver enzymes, and enlargement of the liver and spleen. Kanuma (sebelipase alfa) is an enzyme replacement therapy indicated for individuals with LAL-D.

Kanuma has a boxed warning for risk of anaphylaxis early in the course of therapy, and after extended duration of treatment. Appropriate medical monitoring and support measures, including access to cardiopulmonary resuscitation, should be available during initiation of therapy.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Kanuma (sebelipase alfa)

Requests for initiation of therapy with Kanuma (sebelipase alfa) may be approved if the following criteria are met:

I. Individual is less than 4 years of age; **AND**
II. Individual has a diagnosis of LAL-D disorder; **AND**
III. Documentation is provided that the diagnosis has been ~~confirmed~~ verified by one of the following (Hamilton 2012, OMIM):
   A. A dried blood spot test demonstrating deficient lysosomal acid lipase activity; **OR**
   B. Molecular genetic test shows mutations in the lipase A, lysosomal acid type (LIPA) gene;

OR

IV. Individual is 4 years of age and older (Burton 2015); **AND**
V. Individual has a diagnosis of LAL-D disorder; **AND**
VI. Documentation is provided that the diagnosis has been ~~confirmed~~ verified by one of the following (Hamilton 2012, OMIM):
   A. A dried blood spot test demonstrating deficient lysosomal acid lipase activity; **OR**
   B. Molecular genetic test shows mutations in the LIPA gene;

AND

VII. Documentation is provided that individual has a baseline alanine aminotransferase (ALT) level greater than or equal to 1.5 times the upper limit of normal (Burton 2015).

Continuing treatment with Kanuma (sebelipase alfa) may be approved when the following criteria are met:

I. Individual has a diagnosis of LAL-D disorder; **AND**
II. Documentation is provided that the diagnosis has been verified by one of the following (Hamilton 2012, OMIM):
   A. Pretreatment results from dried blood spot test demonstrated deficient lysosomal acid lipase activity; **OR**
   B. Molecular genetic test shows mutations in the lipase A, lysosomal acid type (LIPA) gene;

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AND

~~I.~~III. Individual has had a clinical improvement in symptoms or lab values.

Requests for Kanuma (sebelipase alfa) may not be approved when the above criteria are not met and for all other indications.

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J2840	Injection, sebelipase alfa, 1 mg [~~KANUMA~~Kanuma]

ICD-10 Diagnosis

E75.5	Other lipid storage disorders (Wolman's disease)
E75.6	Lipid storage disorder, unspecified

Document History

Revised: 03/10/2025
Document History:

- 03/10/2025 – Annual Review: Update continuation criteria to require verification of disorder. Administrative update to add documentation. Wording and formatting changes. Coding Reviewed: No changes.
- 03/11/2024 – Annual Review: No changes. Coding Reviewed: No changes.
- 03/13/2023 – Annual Review: Update criteria to add statement regarding use outside of the clinical criteria. Wording and formatting changes. Coding Reviewed: No changes.
- 03/14/2022 -- Annual Review: No changes. Coding Reviewed: No changes.
- 08/01/2021 – Administrative update to add documentation.
- 03/15/2021 – Annual Review: No changes. Coding Reviewed: No changes.
- 03/16/2020 – Annual Review: Remove reference to WD and CESD in criteria as both are LAL-D disorder. Coding reviewed: No changes.
- 03/18/2019 – Annual Review: Wording and formatting changes. Coding Reviewed: No changes.
- 08/17/2018 – Annual Review: Initial review of DRUG.00093. Update Kanuma PA to modify continuation of therapy requirements for consistency.

References

1. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: February 3, 2025.
2. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
3. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; Updated periodically.
4. Burton BK, Blawani M, Feillet F, et al. A phase 3 trial of sebelipase alfa in lysosomal acid lipase deficiency. N Engl J Med. 2015; 373:1010-1020.
5. Hamilton J, Jones I, Srivastava R, Galloway P. A new method for the measurement of lysosomal acid lipase in dried blood spots using the inhibitor Lalistat 2. Clinica Chimica Acta. 2012; 413(15-16):1207-1210.
6. Online Mendelian Inheritance in Man (OMIM) #278000 Lysosomal Acid Lipase Deficiency. Available from: http://omim.org/entry/278000. Accessed on: February 3, 2025.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.

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