Kanuma (sebelipase alfa)

Override(s)	Approval Duration
Prior Authorization	1 year

Medications
Kanuma (sebelipase alfa)

APPROVAL CRITERIA

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 lysosomal acid lipase deficiency (LAL-D) disorder ; **AND**
III. Documentation is provided that the diagnosis has been 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 lysosomal acid lipase deficiency (LAL-D) disorder ; **AND**
VI. Documentation is provided that the diagnosis has been 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 the 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;

AND

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.

Key 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.