Reference number(s)
4201-A

STANDARD MEDICARE PART B MANAGEMENT

KANUMA (sebelipase alfa)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Kanuma is indicated for the treatment of patients with a diagnosis of Lysosomal Acid Lipase (LAL) deficiency.

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
A. For initial requests: lysosomal acid lipase enzyme assay or genetic testing results supporting diagnosis.
B. Continuation requests: lab values or chart notes documenting a positive response to therapy (e.g., improvement, stabilization, or slowing of disease progression for weight-for-age z-score if exhibiting growth failure, LDL, HDL, triglycerides, or ALT).

III. CRITERIA FOR INITIAL APPROVAL

Lysosomal acid lipase (LAL) deficiency
Authorization of 12 months may be granted for treatment of LAL deficiency when both of the following criteria are met:
A. Diagnosis of LAL deficiency was confirmed by enzyme assay demonstrating a deficiency of lysosomal acid lipase enzyme activity or by genetic testing; AND
B. Member has alanine aminotransferase level (ALT) ≥ 1.5 times the upper limit of normal (based on the age- and gender-specific normal ranges) on two consecutive ALT measurements obtained at least one week apart.

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization of 12 months may be granted when all of the following criteria are met:
A. The member is currently receiving therapy with the requested medication
B. The requested medication is being used to treat an indication enumerated in Section III

C. The member is receiving benefit from therapy (e.g., improvement, stabilization, or slowing of disease progression for weight-for-age z-score if exhibiting growth failure, low-density lipoprotein [LDL], high-density lipoprotein [HDL], triglycerides, or alanine aminotransferase [ALT]).

V. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:

1. The prescribing information for Kanuma.
2. The available compendium
    a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    b. Micromedex DrugDex
    c. American Hospital Formulary Service- Drug Information (AHFS-DI)
    d. Lexi-Drugs
    e. Clinical Pharmacology
3. Lysosomal Acid Lipase Deficiency (Endotext)

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Kanuma are covered.

VI. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for confirming the diagnosis with enzyme assays or genetic testing prior to using Kanuma to treat LAL deficiency can be found in an article by Wilson and Patni. LAL deficiency can be diagnosed by demonstrating deficient LAL enzyme activity, as well as by genetic testing identifying mutations of the LIPA gene. Historically, enzyme activity was measured in cultured fibroblasts, peripheral leukocytes, or liver tissue. A newer method has been developed to determine LAL activity. This method measures LAL activity in dried blood spots (DBS), and uses Lalistat 2, a highly specific inhibitor of LAL. LAL activity is determined by comparing total lipase activity to lipase activity with Lalistat 2.

VII. REFERENCES

1. Kanuma [package insert]. Cheshire, CT: Alexion Pharmaceuticals Inc.; November 2021.
2. Wilson DP, Patni N. Lysosomal Acid Lipase Deficiency. [Updated 2023 Mar 15]. In: Feingold KR, Anawalt B, Blackman MR, et al., editors. Endotext [Internet]. South Dartmouth (MA): MDText.com, Inc.; 2000-. Available from: https://www.ncbi.nlm.nih.gov/books/NBK395569/