

**POLICY:** Enzyme Replacement Therapy – Kanuma™ (sebelipase alfa injection for intravenous use – Alexion Pharmaceuticals)

**DATE REVIEWED:** 04/15/2020

---

### OVERVIEW

Kanuma is human lysosomal acid lipase (LAL) produced in the egg white of genetically engineered chicken via recombinant DNA technology.<sup>1</sup> LAL catalyzes the breakdown of cholesteryl esters to free cholesterol and fatty acids, and the breakdown of triglycerides to glycerol and free fatty acids.

Kanuma is indicated for the treatment of patients with a diagnosis of LAL deficiency.<sup>1</sup>

### Disease Overview

LAL deficiency is a rare lysosomal storage disorder characterized by absent or deficient LAL activity leading to the accumulation of cholesterol and triglycerides in the liver and other organs.<sup>2,3</sup> Patients with LAL deficiency often have dyslipidemias, cardiovascular disease and progressive liver disease.<sup>2</sup> The disorder has a heterogeneous presentation ranging from a rapidly progressive form occurring in infants which leads to death in the first year of life, to a childhood/adult-onset form with milder signs and symptoms. Almost all patients with childhood/adult-onset LAL deficiency have hepatomegaly with elevated liver transaminases and have an increased risk of developing fibrosis and cirrhosis.<sup>3</sup> The diagnosis of LAL deficiency is established by demonstrating deficient LAL activity in blood or by genetic testing.<sup>2,3</sup>

### POLICY STATEMENT

Prior authorization is recommended for medical benefit coverage of Kanuma. Approval is recommended for those who meet the Criteria and Dosing for the listed indication(s). Extended approvals are allowed if the patient continues to meet the Criteria and Dosing. Requests for doses outside of the established dosing documented in this policy will be considered on a case-by-case basis by a clinician (i.e., Medical Director or Pharmacist).

Because of the specialized skills required for evaluation and diagnosis of patients treated with Kanuma as well as the monitoring required for adverse events and long-term efficacy, approval requires Kanuma to be prescribed by or in consultation with a physician who specializes in the condition being treated.

### RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Kanuma is recommended in those who meet the following criteria:

#### FDA-Approved Indications

**1. Lysosomal Acid Lipase Deficiency.** Approve for 1 year if the patient meets the following criteria (A and B):

- A) The diagnosis is established by one of the following (i or ii);
- i. Patient has a laboratory test demonstrating deficient lysosomal acid lipase activity in leukocytes, fibroblasts, or liver tissue; OR
  - ii. Patient has a molecular genetic test demonstrating lysosomal acid lipase gene mutation; AND
-

- B) Kanuma is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

**Dosing.** Each dose must not exceed 3 mg/kg administered intravenously no more frequently than once per week.<sup>1</sup>

---

### CONDITIONS NOT RECOMMENDED FOR APPROVAL

Kanuma has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions.

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

### REFERENCES

1. Kanuma™ injection [prescribing information]. Cheshire, CT: Alexion Pharmaceuticals; December 2015.
2. Reiner Z, Guardamagna O, Nair D, et al. Lysosomal acid lipase deficiency – an under-recognized cause of dyslipidaemia and liver dysfunction. *Atherosclerosis*. 2014;235:21-30.
3. Erwin AL. The role of sebelipase alfa in the treatment of lysosomal acid lipase deficiency. *Ther Adv Gastroenterol*. 2017;10:553-562.

### HISTORY

Type of Revision	Summary of Changes	Date Reviewed
New Policy	--	04/17/2019
Annual Revision	No criteria changes.	04/15/2020