

Kanuma® (sebelipase alfa)

When requesting Kanuma® (sebelipase alfa), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved Indication

Kanuma (sebelipase alfa) is indicated for the treatment of individuals with lysosomal acid lipase (LAL) deficiency.

Coverage Guidelines

Lysosomal Acid Lipase Deficiency

The individual must meet all of the following criteria for approval:

- Diagnosis is confirmed by one of the following:
 - Laboratory test demonstrating deficient lysosomal acid lipase activity in leukocytes, fibroblasts, or liver tissue; OR
 - Molecular genetic test demonstrating biallelic pathogenic or likely pathogenic lysosomal acid lipase gene variants;
- Kanuma is prescribed by or in consultation with a geneticist, an endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Approval duration: 12 months

Dosing Recommendations

Infants with Rapidly Progressive LAL Deficiency Presenting within the First 6 Months of Life:

- The recommended starting dosage is 1 mg/kg as an intravenous infusion once weekly.
- For patients with a suboptimal clinical response, increase the dosage to 3 mg/kg once weekly.
- For patients with continued suboptimal clinical response, further increase the dosage to 5 mg/kg once weekly.

Pediatric and Adult Patients with LAL Deficiency:

- The recommended dosage is 1 mg/kg as an intravenous infusion once every other week.
- For patients with a suboptimal clinical response, increase the dosage to 3 mg/kg once every other week.

References

1. Kanuma™ injection [prescribing information]. Cheshire, CT: Alexion Pharmaceuticals; November 2021.
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.