

## Kanuma® (sebelipase alfa) Injection

When requesting Kanuma® (sebelipase alfa) injection, the individual requiring treatment must be diagnosed with an 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 lysosomal acid lipase gene mutation;
- 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.