

# Specialty Guideline Management

## Kanuma

### Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name    |
|------------|-----------------|
| Kanuma     | sebelipase alfa |

### 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 Indications<sup>1</sup>

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

All other indications are considered experimental/investigational and not medically necessary.

### Documentation

Submission of the following information is necessary to initiate the prior authorization review:

#### Initial Requests:

- Lysosomal acid lipase enzyme assay or genetic testing results supporting diagnosis.
- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g., hepatomegaly, splenomegaly, poor growth/failure to thrive, dyslipidemia).

#### 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, low-density lipoprotein [LDL], high-density lipoprotein [HDL], triglycerides, or alanine aminotransferase [ALT]).

## Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

## Coverage Criteria

### Lysosomal Acid Lipase (LAL) Deficiency<sup>1-3</sup>

Authorization of 12 months may be granted for treatment of LAL deficiency when all of the following criteria are met:

- Diagnosis of LAL deficiency was confirmed by enzyme assay demonstrating a deficiency of lysosomal acid lipase enzyme activity or by genetic testing.
- Member has alanine aminotransferase level (ALT) greater than or equal to 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.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g., hepatomegaly, splenomegaly, poor growth/failure to thrive, dyslipidemia).

## Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria who are responding to 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]).

## References

1. Kanuma [package insert]. Boston, MA: Alexion Pharmaceuticals, Inc.; July 2024.
2. Burton BK, Balwani, M, Feillet F, et al. A Phase 3 Trial of Sebelipase Alfa in Lysosomal Acid Lipase Deficiency. *N Engl J Med* 2015; 373:1010-20.
3. Balwani M, Vijay S. Lysosomal Acid Lipase Deficiency. 2015 Jul 30 [Updated 2026 Jan 15]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. *GeneReviews*® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK305870/>