

Reference number
2094-A

# SPECIALTY GUIDELINE 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 are considered experimental/investigational and not medically necessary.

#### II. DOCUMENTATION

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

- A. 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)  $\geq 1.5$  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

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for lysosomal acid lipase (LAL) deficiency 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]).

#### V. REFERENCES

1. Kanuma [package insert]. Cheshire, CT: Alexion Pharmaceuticals Inc.; December 2015.
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.