Effective Date: 12/2017
Revised: 11/2018, 12/2019 Reviewed: 12/2017, 11/2018, 12/2019 Scope: Medicaid

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 Indications
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 are not a covered benefit.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the initial prior authorization review: enzyme assay demonstrating a deficiency of lysosomal acid lipase enzyme activity or genetic testing revealing mutation in the lipase A, lysosomal acid type (LIPA) gene. For both initial and continuation of therapy, recent liver function tests, lipid panel, and weight are required.

III. CRITERIA FOR INITIAL APPROVAL

Lysosomal acid lipase (LAL) deficiency
An authorization of 6 months may be granted for patients at least 1 month old for the treatment of LAL deficiency when all of the following criteria are met:

1. The diagnosis of LAL deficiency was confirmed by enzyme assay demonstrating a deficiency of lysosomal acid lipase enzyme activity or by genetic testing
2. Prescribing physician is a specialist in genetics and metabolism
3. Baseline liver function and lipid panel provided
4. For adults and pediatric patients greater than 6 months of age, dose requested does not exceed 1 mg/kg every other week.
5. For pediatric patients 1 to 6 months of age with rapidly progressive disease, dose requested does not exceed 3mg/kg once weekly.

IV. CONTINUATION OF THERAPY

An authorization of 6 months may be granted for treatment of LAL deficiency when all of the following criteria are met:

1. Patient continues to meet the initial criteria in section III

2. Patient is tolerating treatment
3. Documented clinical benefit with therapy (e.g. reduction in LDL, triglycerides, AST or ALT, increase in HDL, improvement in weight-for-age z-scores, reduction in liver fat content)

V. REFERENCES

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