Protocol for Kanuma® (sebelipase alfa)

Approved October 2023

Background: *Lysosomal acid lipase deficiency (LAL-D) is a rare, autosomal recessive lysosomal storage disorder associated with functional mutations in the LAL gene (LIPA) that cause a deficiency or absence of LAL activity. It is characterized by intracellular accumulation of cholesteryl esters and triglycerides (TGs) and multisystem involvement.*

Kanuma *is a hydrolytic lysosomal cholesteryl ester and triacylglycerol-specific enzyme indicated for the treatment of patients with a diagnosis of LAL-D.*

Criteria for approval:

1. Patient is 1 month or older; **AND**

2. Patient has a diagnosis of LAL-D; **AND**

3. Diagnosis of LAL-D is confirmed by:
   a. Enzyme assay demonstrating deficiency of LAL activity; **OR**
   b. Documented molecular genetic test showing mutations in the lysosomal acid type (LIPA) gene

4. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Continuation of therapy:

1. Patient is responding positively to therapy as evidenced by documentation of clinical response which may include:
   a. For members with rapidly progressive disease presenting within first 6 months of life: continued survival
   b. There is improvement in other parameters related to LAL deficiency, including decrease in low-density lipoprotein cholesterol (LDL-C), non-high-density lipoprotein cholesterol (non-HDL-C), or triglycerides; increase in HDL-C, etc.

2. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

References:

1. Kanuma [prescribing information]. Alexion Pharmaceuticals Inc. Cheshire, CT 06410 April 2015
2. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2020. Updated periodically
3. Burton BK, Feillet F, et al: Sebelipase alfa in children and adults with lysosomal acid lipase deficiency: Final results of the ARISE study. Journal of Hepatology 2022 vol. 76:577-587
4. Pastores GM, Hughes DA. Lysosomal Acid Lipase Deficiency: Therapeutic Options. Drug Design, Development and Therapy 2020:14 591-601