Clinical Policy: Sebelipase Alfa (Kanuma)

Reference Number: PA.CP.PHAR.159
Effective Date: 01/18
Last Review Date: 04/18 Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for sebelipase alfa (Kanuma™)

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

Policy/Criteria
It is the policy of health plans affiliated with Pennsylvania Health and Wellness that Kanuma is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Lysosomal Acid Lipase Deficiency (must meet all):

1. Diagnosis of lysosomal acid lipase (LAL) deficiency confirmed by one of the following:
   a. Enzyme assay demonstrating a deficiency of LAL activity;
   b. LIPA gene mutation.
2.
3. Dose does not exceed 1 mg/kg every other week (*1 mg/kg/week for members with rapidly progressive disease presenting within first 6 months of life; may be increased to 3 mg/kg/week upon documentation of suboptimal clinical response to 1 mg/kg/week*).

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Lysosomal Acid Lipase Deficiency (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy as evidenced by documentation of clinical response which may include, but is not limited to:
   a. For members with rapidly progressive disease presenting within first 6 months of life: continued survival;
   b. For all other members: decrease in low-density lipoprotein cholesterol [LDL-c], non-high-density lipoprotein cholesterol [non-HDL-c], or triglycerides; increase in HDL-c; normalization of alanine aminotransferase [ALT] or aspartate

aminotransferase [AST]; reduction in hepatic fat content, steatosis, or liver volume;

3. If request is for a dose increase, new dose does not exceed 1 mg/kg every other week (*1 mg/kg/week for members with rapidly progressive disease presenting within first 6 months of life; may be increased to 3 mg/kg/week upon documentation of suboptimal clinical response to 1 mg/kg/week*).

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or
2. Refer to PA.CP.PHAR.57 - Global Biopharm Policy**.**

Background

Description/Mechanism of Action:

LAL deficiency is an autosomal recessive lysosomal storage disorder characterized by a genetic defect resulting in a marked decrease or loss in activity of the lysosomal acid lipase (LAL) enzyme. The primary site of action of the LAL enzyme is the lysosome, where the enzyme normally causes the breakdown of lipid particles including LDL-c. Deficient LAL enzyme activity results in progressive complications due to the lysosomal accumulation of cholesteryl esters and triglycerides in multiple organs, including the liver, spleen, intestine, and the walls of blood vessels. The resulting lipid accumulation in the liver may lead to increased liver fat content and progression of liver disease, including fibrosis and cirrhosis. Lipid accumulation in the intestinal wall leads to malabsorption and growth failure. In parallel, dyslipidemia due to impaired degradation of lysosomal lipid is common with elevated LDL-c and triglycerides and low HDL-cholesterol (HDL-c). Sebelipase alfa binds to cell surface receptors via glycans expressed on the protein and is subsequently internalized into lysosomes. Sebelipase alfa catalyzes the lysosomal hydrolysis of cholesteryl esters and triglycerides to free cholesterol, glycerol and free fatty acids.

Formulations:

Kanuma (sebelipase alfa): Solution for intravenous use

- 20 mg/10 mL vial; 2 mg/mL (195 to 345 units/mg)

Appendices

Appendix A: Abbreviation Key

HDL-c: High density lipoprotein cholesterol
LAL: Lysosomal acid lipase
LDL-c: Low density lipoprotein cholesterol
rhLAL: Recombinant human lysosomal acid lipase

Reviews, Revisions, and Approvals	Date	Approval Date
2Q 2018 annual review: Added age restriction and max dose criteria. Added examples of what may constitute positive response to therapy; references reviewed and updated.	02.26.18	

References

1. Kanuma prescribing information. Cheshire, CT: Alexion Pharmaceuticals, Inc.; Cambridge, MA: Genzyme Corporation; December 2015. Available at http://www.kanuma.com/docs/full-prescribing-information.pdf. Accessed February 26, 2018.
2. Zhang B, Porto AF. Cholesteryl ester storage disease: Protean presentations of lysosomal acid lipase deficiency. J Pediatr Gastroenterol Nutr. 2013; 56(6): 682.