BlueCross BlueShield Federal Employee Program

KANUMA
(sebelipase alfa)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Kanuma (sebelipase alfa) is used to treat Lysosomal Acid Lipase (LAL) deficiency. LAL deficiency is a disorder characterized by a genetic defect resulting in a marked decrease or loss in activity of the lysosomal acid lipase enzyme. This enzyme normally causes the breakdown of lipid particles including LDL cholesterol (LDL-c). Deficient LAL enzyme activity results in progressive complications due to the lysosomal accumulation of fat molecules 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. Accumulation of lipid 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). Kanuma is administered via intravenous infusions once weekly or once every other week (1).

Regulatory Status

FDA-approved indication: Kanuma is a hydrolytic lysosomal cholesteryl ester and triacylglycerol-specific enzyme indicated for the treatment of patients with a diagnosis of Lysosomal Acid Lipase (LAL) deficiency (1).

Kanuma contains a boxed warning for hypersensitivity reactions, including anaphylaxis. If a severe hypersensitivity reaction occurs, discontinue Kanuma and immediately initiate appropriate medical treatment, including use of epinephrine (1).

Safety and effectiveness of Kanuma have been established in pediatric patients aged 1 month and older (1).

Summary

Kanuma is indicated for patients with a diagnosis of Lysosomal Acid Lipase (LAL) deficiency. LAL deficiency is a disorder characterized by a genetic defect resulting in a marked decrease or loss in activity of the lysosomal acid lipase enzyme. This enzyme normally causes the breakdown of lipid particles including LDL cholesterol. Kanuma is administered via intravenous infusions once weekly or once every other week. Kanuma has a boxed warning for hypersensitivity reactions. Safety and effectiveness of Kanuma have been established in pediatric patients aged 1 month and older (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Kanuma while maintaining optimal therapeutic outcomes.

References

1. Kanuma [package insert]. New Haven, CT: Alexion Pharmaceuticals Inc.; July 2024.