PHARMACY POLICY STATEMENT
Michigan Medicaid

DRUG NAME	Kanuma (sebelipase alfa)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Kanuma, approved by the FDA in 2015, is a recombinant human lysosomal acid lipase (a lysosomal enzyme) indicated for the treatment of Lysosomal Acid Lipase (LAL) deficiency.
LAL deficiency is a lysosomal storage disorder caused by a genetic defect of the LIPA gene that results in decreased or absent activity of the LAL enzyme. 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. Accumulation of lipids in the liver can lead to fibrosis and cirrhosis. Lipid accumulation in the intestinal walls can lead to malabsorption and growth failure. Dyslipidemia and accelerated atherosclerosis also occur.
Kanuma works as an enzyme replacement therapy (ERT) and catalyzes the lysosomal hydrolysis of cholesteryl esters and triglycerides to break them down. It is the first drug to be approved for LAL-D and the first treatment to target the underlying pathology. Previous management focused on strategies like lipid lowering medications, diet modification, liver transplant, or stem cell transplant.
Wolman disease is the early infantile-onset phenotype of LAL-D which is more severe and rapidly progressive, with survival not expected beyond infancy. Kanuma significantly prolongs the life expectancy of these patients. Cholesterol ester storage disease (CESD) has a more variable phenotype with onset during childhood or adulthood. Kanuma has proven benefits for these patients as well.

Kanuma (sebelipase alfa) will be considered for coverage when the following criteria are met:

Lysosomal Acid Lipase (LAL) Deficiency

For **initial** authorization:

1. Member is at least 1 month of age; AND
2. Medication must be prescribed by or in consultation with a hepatologist, cardiologist, metabolic specialist, or geneticist; AND
3. Member has a diagnosis of LAL deficiency confirmed by at least one of the following methods:
   a) Deficient LAL enzyme activity (assay)
   b) Identification of biallelic pathogenic mutations of the LIPA gene (genetic testing); AND
4. Member demonstrates at least one clinical feature of LAL-D such as dyslipidemia, elevated transaminases (ALT, AST), impaired growth or failure to thrive, malabsorption, hepatomegaly, adrenal calcification (in Wolman's disease), or advanced liver disease.
5. **Dosage allowed/Quantity limit:**
   Infants with rapidly progressive LAL-D within first 6 months of life: Starting dose of 1 mg/kg once weekly via IV infusion. If clinical response is suboptimal, may increase to 3 mg/kg once weekly, and further to 5 mg/kg once weekly if needed.

Pediatric and adult LAL-D: 1 mg/kg IV infusion every other week. If clinical response is suboptimal, may increase to 3 mg/kg every other week.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must document a positive clinical response to treatment such as improved lipid profile (LDL-c, triglycerides), liver biomarkers (ALT, AST), liver volume, or growth/weight z-scores (if pediatric).

If all the above requirements are met, the medication will be approved for an additional 12 months.

HAP CareSource considers Kanuma (sebelipase alfa) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
04/11/2018	New policy for Kanuma created.
01/10/2022	Transferred to new template. Updated references. Corrected max dose from 3 mg/kg to 5 mg/kg; added ped/adult dosing section which was missing. Changed prescribed by to by or in consultation with; also removed general specialist, added geneticist. Amended age minimum from 8 months to 1 month. Added tests for diagnostic confirmation. Removed all criteria that listed manifestations from a specific clinical trial and was divided by age; replaced with general list of manifestations. Specified renewal criteria.
04/18/2024	Annual review; no updates.
05/15/2026	Updated references.

References:

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5. Demaret T, Lacaille F, Wicker C, et al. Sebelipase alfa enzyme replacement therapy in Wolman disease: a nationwide cohort with up to ten years of follow-up. *Orphanet J Rare Dis*. 2021;16(1):507. Published 2021 Dec 14. doi:10.1186/s13023-021-02134-3
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8. de Las Heras J, Almohalla C, Blasco-Alonso J, et al. Practical Recommendations for the Diagnosis and Management of Lysosomal Acid Lipase Deficiency with a Focus on Wolman Disease. *Nutrients*. 2024;16(24):4309. Published 2024 Dec 13. doi:10.3390/nu16244309

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