Pharmacy Medical Necessity Guidelines: Kanuma™ (sebelipase alfa)

Effective: June 15, 2020

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	MED	Department to Review	PRECERT /MM

These pharmacy medical necessity guidelines apply to the following:

Commercial Products

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Tufts Health Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

Commercial Products:
PRECERT: 617.972.9409

Tufts Health Public Plans Products:
MM: 888.415.9055

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS

Kanuma (sebelipase) 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.

Lysosomal Acid Lipase deficiency is an autosomal recessive lysosomal storage disorder characterized by a genetic defect (mutation in the LIPA gene) resulting in a marked decrease or loss in activity of the LAL enzyme. The primary site of action of the LAL enzyme is the lysosome, where the enzyme normally causes the breakdown of lipid particles. 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.

LAL deficiency is sub-classified as Wolman disease in infants and cholesteryl ester storage disease (CESD) in children and adults. Wolman disease, which affects one to two infants per million births, is a rapidly progressive disease characterized by a loss of LAL and presents during first weeks to months of life. Patients rarely live beyond 6 months to one year of age due to multi-organ failure. A mild, later-onset form of LAL-D presents in both pediatric and adult patients as CESD with a partial loss of LAL. CESD affects one individual per 40,000 births and patients have life expectancy that is highly dependent on the severity and extent of clinical complications. On average, patients begin experiencing symptoms at five years of age (with latest documented cases being 44 years of age for men and 68 years of age in women), in both male and female patients.

LAL deficiency is often underdiagnosed due to its common clinical presentation and combination of clinical features relative to other conditions. Common features at diagnosis of Lysosomal Acid Lipase deficiency include liver dysfunction and hepatomegaly, which can occur early in patients and progress to fibrosis, cirrhosis, and liver failure. Dyslipidemia is a common, with elevated serum cholesterol markers such as LDL-C and triglyceride (TG) that can lead to cardiovascular disease. Patients may also experience elevated transaminases (e.g., alanine aminotransferase test [ALT] and aspartate aminotransferase test [AST]), which are early asymptomatic indicators of liver damage.

The hallmarks of Wolman's disease includes failure to thrive, malnutrition, adrenal gland calcification, liver failure (patients may experience jaundice and cachexia), hepatosplenomegaly (seen with abdominal distension) and other GI symptoms, such as diarrhea and vomiting. The gastrointestinal and growth failure symptoms are often the first observed. Diagnosis cannot be based solely on signs and symptoms of LAL deficiency (such as liver damage), as these become less explicit with pediatric and adult patients and not seen as rapidly as they would be among infants. The dried blood spot test

is the most easily accessible, acute and low-cost assay screening tool that can replace the need for extensive complete sequencing of LIPA to diagnose LAL deficiency. If diagnosed early, disease-associated morbidity and mortality can be reduced.

COVERAGE GUIDELINES

The plan may authorize coverage of Kanuma (sebelipase alfa) for Members, when all of the following criteria are met:

1. Documented diagnosis of one of the following forms of Lysosomal Acid Lipase deficiency:
   a) Wolman disease
   b) Cholesteryl ester storage disease with elevated alanine aminotransferase levels at least 1.5 times the upper limit of the normal range reported by the laboratory

AND

2. Diagnosis has been confirmed by a dried blood spot test, genetic testing, or leucocyte testing

AND

3. The prescribing physician is a specialist in genetics and metabolism

LIMITATIONS

None

CODES

The following HCPCS/CPT code(s) are:

Code	Description
J2840	Injection, sebelipase alfa, 1 mg

REFERENCES

1. Burton BK, Balwani M, Feillet F et al. A phase 3 trial of sebelipase alfa in lysosomal acid lipase deficiency. *N Engl J Med*. 2015; 373:1010-20.
2. Burton BK, Deegan PB, Enns GM et al. Clinical features of lysosomal acid lipase deficiency. *J Pediatr Gastroenterol Nut*. 2015; Dec;61 (6):619-25
3. Hoffman EP, Barr ML, Giovanni MA, Murray MF. Lysosomal acid lipase deficiency. *GeneReviews®*. 2015 Jul 30. URL: ncbi.nlm.nih.gov/books/NBK305870/?report=printable. Available from Internet. Accessed 2016 April 18.
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6. Kanuma (sebelipase afla) [prescribing Information]. Chesire, CT: Alexion Pharmaceuticals Inc.; December 2015.
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10. Porto AF. Lysosomal acid lipase deficiency: diagnosis and treatment of Wolman and Cholesteryl Ester Storage Diseases. *Pediatr Endocrinol Rev*. 2014 Sep;12 Suppl 1:125-32.
11. Reiner Z, Guardamagna O, Nair D. Lysosomal acid lipase deficiency – an under-recognized cause of dyslipidemia and liver dysfunction. *Atherosclerosis*. 2014 Jul;235(1):21-30.

APPROVAL HISTORY

May 10, 2016: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. July 1, 2016: Administrative Update: Added HCPCS code to Medical Necessity Guideline.
2. January 1, 2017: Administrative update: added new J code (J2840) to Medical Necessity Guideline and removed expired C code (C9478).

3. April 11, 2017: Administrative update. Effective 6/1/2017, Medical Necessity Guideline applies to Tufts Health RITogether.
4. May 9, 2017: No changes.
5. June 12, 2018: No changes.
6. March 12, 2019: No changes.
7. June 9, 2020: Removed reauthorization criteria from Medical Necessity Guideline.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.