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Coverage Policy Number IP0448

Sebelipase Alfa

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Related Coverage Resources

INSTRUCTIONS FOR USE
The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

Overview

This policy supports medical necessity review for sebelipase alfa intravenous infusion (**Kanuma**®).

Receipt of sample product does not satisfy any criteria requirements for coverage.

Medical Necessity Criteria

Sebelipase alfa (Kanuma) is considered medically necessary when the following are met:

1. **Lysosomal Acid Lipase Deficiency.** Individual meets **BOTH** of the following criteria (A and B):
   A. The diagnosis is established by one of the following (i or ii):
      i. Individual has a laboratory test demonstrating deficient lysosomal acid lipase activity in leukocytes, fibroblasts, or liver tissue
      ii. Individual has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic lysosomal acid lipase (*LAL*) gene variants

B. The medication is prescribed by, or in consultation with, a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

When coverage is available and medically necessary, the dosage, frequency, duration of therapy, and site of care should be reasonable, clinically appropriate, and supported by evidence-based literature and adjusted based upon severity, alternative available treatments, and previous response to therapy.

Reauthorization Criteria

Sebelipase alfa (Kanuma) is considered medically necessary for continued use when initial criteria are met AND there is documentation of beneficial response.

Authorization Duration

Initial and reauthorization approval duration: up to 12 months

Conditions Not Covered

Any other use is considered experimental, investigational, or unproven.

Coding Information

Note:

1) This list of codes may not be all-inclusive.
2) Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

Considered Medically Necessary when criteria in the applicable policy statements listed above are met:

HCPCS Codes	Description
J2840	Injection, sebelipase alfa, 1 mg

Background

OVERVIEW

Kanuma, a human lysosomal acid lipase (LAL), indicated for the treatment of patients with a diagnosis of **LAL deficiency**.[1] It is produced in the egg white of genetically engineered chickens via recombinant DNA technology. LAL catalyzes the breakdown of cholesteryl esters to free cholesterol and fatty acids, and the breakdown of triglycerides to glycerol and free fatty acids.

Disease Overview

LAL deficiency is a rare lysosomal storage disorder characterized by absent or deficient LAL activity leading to the accumulation of cholesterol and triglycerides in the liver and other organs.[2,3] Patients with LAL deficiency often have dyslipidemias, cardiovascular disease and progressive liver disease.[2] The disorder has a heterogeneous presentation ranging from a rapidly progressive form occurring in infants which leads to death in the first year of life, to a childhood/adult-onset form with milder signs and symptoms. Almost all patients with childhood/adult-onset LAL deficiency have hepatomegaly with elevated liver transaminases and have an increased risk of developing fibrosis and cirrhosis.[3] The diagnosis of LAL deficiency is established by demonstrating deficient LAL activity in leukocytes, fibroblasts, or liver tissue; or by genetic testing.[2,3]

Dosing and Availability

Infants with Rapidly Progressive LAL Deficiency Presenting within the First 6 Months of Life:

- The recommended starting dosage is 1 mg/kg administered as an intravenous infusion once weekly.
- For patients with a suboptimal clinical response, increase the dosage to 3 mg/kg once weekly.
- For patients with continued suboptimal clinical response on the 3 mg/kg once weekly dosage, further increase the dosage to 5 mg/kg once weekly.
- A suboptimal clinical response is defined as any of the following: poor growth, deteriorating biochemical markers, or persistent or worsening organomegaly.

Pediatric and Adult Patients with LAL Deficiency:

- The recommended dosage is 1 mg/kg administered as an intravenous infusion once every other week.
- For patients with a suboptimal clinical response, increase the dosage to 3 mg/kg once every other week.
- A suboptimal clinical response is defined as any of the following: poor growth, deteriorating biochemical markers [e.g., alanine aminotransferase (ALT), aspartate aminotransferase (AST)], and/or parameters of lipid metabolism [e.g., low-density lipoprotein cholesterol (LDL-c), triglycerides (TG)].

Sebelipase Alfa is supplied as an injection in a 20 mg/10 mL (2 mg/mL) solution in single-dose, glass vials.

References

1. Kanuma® intravenous infusion [prescribing information]. Cheshire, CT: Alexion; November 2021.
2. Reiner Z, Guardamagna O, Nair D, et al. Lysosomal acid lipase deficiency – an under-recognized cause of dyslipidaemia and liver dysfunction. *Atherosclerosis*. 2014;235:21-30.
3. Erwin AL. The role of sebelipase alfa in the treatment of lysosomal acid lipase deficiency. *Ther Adv Gastroenterol*. 2017;10:553-562.