Medical Policy:

Kanuma™ (sebelipase) alfa intravenous

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.313	March 27, 2025	March 2019

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The treating physician or primary care provider must submit to EmblemHealth, or ConnectiCare, as applicable (hereinafter jointly referred to as "EmblemHealth"), the clinical evidence that the member meets the criteria for the treatment or surgical procedure. Without this documentation and information, EmblemHealth will not be able to properly review the request preauthorization or post-payment review. The clinical review criteria expressed below reflects how EmblemHealth determines whether certain services or supplies are medically necessary. This clinical policy is not intended to pre-empt the judgment of the reviewing medical director or dictate to health care providers how to practice medicine. Health care providers are expected to exercise their medical judgment in rendering appropriate care. Health care providers are expected to exercise their medical judgment in rendering appropriate care.

EmblemHealth established the clinical review criteria based upon a review of currently available clinical information (including clinical outcome studies in the peer reviewed published medical literature, regulatory status of the technology, evidence-based guidelines of public health and health research agencies, evidence-based guidelines and positions of leading national health professional organizations, views of physicians practicing in relevant clinical areas, and other relevant factors). EmblemHealth expressly reserves the right to revise these conclusions as clinical information changes and welcomes further relevant information. Each benefit program defines which services are covered. The conclusion that a particular service or supply is medically necessary does not constitute a representation or warranty that this service or supply is covered and/or paid for by EmblemHealth, as some programs exclude coverage for services or supplies that EmblemHealth considers medically necessary.

If there is a discrepancy between this guideline and a member's benefits program, the benefits program will govern. Identification of selected brand names of devices, tests and procedures in a medical coverage policy is for reference only and is not an endorsement of any one device, test or procedure over another. In addition, coverage may be mandated by applicable legal requirements of a state, the Federal Government or the Centers for Medicare & Medicaid Services (CMS) for Medicare and Medicaid members. All coding and web site links are accurate at time of publication.

EmblemHealth may also use tools developed by third parties, such as the MCG™ Care Guidelines, to assist us in administering health benefits. The MCG™ Care Guidelines are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice. EmblemHealth Services Company, LLC, has adopted this policy in providing management, administrative and other services to EmblemHealth Plan, Inc., EmblemHealth Insurance Company, EmblemHealth Services Company, LLC, and Health Insurance Plan of Greater New York (HIP) related to health benefit plans offered by these entities. ConnectiCare, an EmblemHealth company, has also adopted this policy. All of the aforementioned entities are affiliated companies under common control of EmblemHealth Inc.

Definitions

Kanuma, a human lysosomal acid lipase (LAL), indicated for the treatment of patients with a diagnosis of LAL deficiency. 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.

Length of Authorization

Coverage will be provided for 12 months

Dosing Limits [Medical Benefit]

Each dose must not exceed 5 mg/kg administered intravenously no more frequently than once per week.

Max Units (per dose and over time) [HCPCS Unit]:

- 340 billable units every 14 days

Guideline

I. INITIAL CRITERIA

1. **Lysosomal Acid Lipase Deficiency.** Approve for 1 year if the patient meets the following criteria (A and B):
   A. The diagnosis is established by one of the following (i or ii):
      i. Patient has a laboratory test demonstrating deficient lysosomal acid lipase activity in leukocytes, fibroblasts, or liver tissue; **OR**
      ii. Patient has a molecular genetic test demonstrating demonstrating biallelic pathogenic or likely pathogenic lysosomal acid lipase (*LAL*) gene variants**; AND**
   B. Documented baseline values for one or more of the following have been obtained:
      i. Weight-for-age z-scores for patients exhibiting growth failure
      ii. Low-density lipoprotein cholesterol (LDL-c)
      iii. High-density lipoprotein cholesterol (HDL-c)
      iv. Non-HDL-c
      v. Triglycerides
      vi. Aspartate aminotransferase (AST) and/or alanine aminotransferase (ALT); **AND**
   C. Kanuma 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.

II. RENEWAL CRITERIA

Coverage can be renewed based upon the following criteria:

1. Patient continues to Initial Criteria; **AND**

2. Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: hypersensitivity reactions including anaphylaxis, etc.; **AND**

A. Treatment has resulted in clinical benefit as evidenced in one or more of the following:
   i. Improvement in weight-for-age z-scores for patients exhibiting growth failure
   ii. Improvement in LDL-c
   iii. Improvement in HDL-c
   iv. Improvement in non-HDL-c
   v. Improvement in triglycerides
   vi. Improvement of AST and/or ALT; **OR**

B. Dose escalation** in pediatric and adult patients with a suboptimal clinical response to the 1 mg/kg dose defined by at least one of the following:
   i. Poor growth
   ii. Deteriorating biochemical markers (e.g., ALT, AST), and/or parameters of lipid metabolism (e.g., LDL-c, triglycerides); **OR**

C. Dose escalation** for infants with rapidly progressive disease presenting within the first 6 months of life who have a suboptimal clinical response to the 1 mg/kg dose or 3 mg/kg dose defined by at least one of the following:
   i. Poor growth
   ii. Deteriorating biochemical markers (e.g., ALT, AST)
   iii. Persistent or worsening organomegaly

May increase dose to 3 mg/kg once every other week for patients who do not achieve an optimal clinical response to the 1 mg/kg dose

Applicable Procedure Codes

Code	Description
J2840	Injection, sebelipase alfa, 1 mg

Applicable NDCs

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Code	Description
25682-0007-01	Kanuma 20mg/10mL Solution

ICD-10 Diagnoses

Code	Description
E75.5	Other lipid storage disorders

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	03/27/2025	Annual Review: Initial Criteria: Updated the following statement to remove: "lysosomal acid lipase gene mutation; AND " revised as: "Patient has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic lysosomal acid lipase (*LAL*) gene variants; AND" Added: " Documented baseline values for one or more of the following have been obtained: Weight-for-age z-scores for patients exhibiting growth failure Low-density lipoprotein cholesterol (LDL-c) High-density lipoprotein cholesterol (HDL-c) Non-HDL-c Triglycerides Aspartate aminotransferase (AST) and/or alanine aminotransferase (ALT); AND" RENEWAL CRITERIA Added: "Coverage can be renewed based upon the following criteria: Patient continues to Initial Criteria; AND Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: hypersensitivity reactions including anaphylaxis, etc.; AND Treatment has resulted in clinical benefit as evidenced in one or more of the following: Improvement in weight-for-age z-scores for patients exhibiting growth failure Improvement in LDL-c Improvement in HDL-c Improvement in non-HDL-c Improvement in triglycerides Improvement of AST and/or ALT; OR Dose escalation** in pediatric and adult patients with a suboptimal clinical response to the 1 mg/kg dose defined by at least one of the following: Poor growth Deteriorating biochemical markers (e.g., ALT, AST), and/or parameters of lipid metabolism (e.g., LDL-c, triglycerides); OR Dose escalation** for infants with rapidly progressive disease presenting within the first 6 months of life who have a suboptimal clinical response to the 1 mg/kg dose or 3 mg/kg dose defined by at least one of the following: Poor growth Deteriorating biochemical markers (e.g., ALT, AST) Persistent or worsening organomegaly" Updated dosing limits.
EmblemHealth & ConnectiCare	2/15/2024	Annual Review: Updated dosing limits, no criteria changes
EmblemHealth & ConnectiCare	04/07/2023	Transfer from CCUM template to Co-Branded Medical Template Retired MG.MM.PH.154
EmblemHealth & ConnectiCare	04/06/2022	Annual Revision: Lysosomal Acid Lipase Deficiency: The dosing limit was increased from 3 mg/kg to 5 mg/kg due to revisions in the prescribing information.
EmblemHealth & ConnectiCare	04/07/2021	Annual Revision: no criteria changes

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References

1. Kanuma™ intravenous infusion [prescribing information]. Cheshire, CT: Alexion; November 2021.

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