Blue Cross Blue Shield Blue Care Network of Michigan

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Medical benefit drug policies are a source for BCBSM and BCN medical policy information only. These documents are not to be used to determine benefits or reimbursement. Please reference the appropriate certificate or contract for benefit information. This policy may be updated and therefore subject to change.

Effective Date: 10/03/2024

Kanuma™ (sebelipase alfa)

HCPCS: J2840

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

A. Coverage of the requested drug is provided when all the following are met:
   a. FDA approved indication
   b. FDA approved age
   c. Confirmation of diagnosis by serum assay showing a decrease of lysosomal acid lipase (LAL) activity followed by genetic testing showing a mutation in the LIPA gene
   d. Symptomatic manifestations of the disease are present, such as, elevated liver enzymes, microvesicular steatosis, elevated low-density lipoprotein, low high-density lipoprotein, or coronary artery disease
   e. Trial and failure, contraindication, OR intolerance to the preferred drugs as listed in BCBSM/BCN's utilization management medical drug list.

B. Quantity Limitations, Authorization Period and Renewal Criteria
   a. Quantity Limits: Align with FDA recommended dosing
   b. Authorization Period: 1 year at a time
   c. Renewal Criteria: Clinical documentation must be provided to confirm that current criteria are met and that the medication is providing clinical benefit

***Note: Coverage and approval duration may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at http://www.cms.hhs.gov/. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Background Information:

- Lysosomal acid lipase deficiency (LAL-D) is an autosomal recessive lysosomal storage disorder caused by a mutation on the LIPA gene. It is characterized by accumulation of undegraded triglycerides and cholesteryl esters due to a deficiency or insufficient activity of the enzyme lysosomal acid lipase (LAL). Age of onset and phenotypic spectrum are variable and range from an infantile-onset form or Wolman disease with severe clinical course and death before 1 year of age to childhood/adult-onset disease with milder symptoms, historically also known as cholesteryl ester storage disease. The most common symptoms of Wolman's disease include abdominal distension, hepatosplenomegaly, ascites, fibrosis of the liver, vomiting, diarrhea, steatorrhea, malnutrition, failure to thrive, calcification of the adrenal gland, and developmental delays. Patients with cholesteryl ester storage disease present with a range of symptoms depending on the degree of LAL activity including hypercholesterolemia, hypertriglyceridemia, high-density lipoprotein deficiency, abnormal lipid deposits, hepatomegaly, splenomegaly, adrenomegaly, fatty liver disease, and liver fibrosis.

- LAL-D diagnosis is confirmed through identifying reduced lysosomal acid lipase activity in peripheral leukocytes or skin fibroblasts followed by genetic testing that shows the patient has a mutation of the LIPA gene.

- Enzyme replacement is the standard of care in lysosomal acid lipase deficiency. Kanuma is the only enzyme replacement therapy FDA approved for the treatment of pediatric and adult patients with LAL-D. Kanuma has been studied in patients as young as 1 month of age. All patients studied were symptomatic at study entry and showed improvement in liver function, lipid panel, hepatosplenomegaly, and gastrointestinal symptoms.

References:

1. Kanuma [prescribing information]. New Haven, CT: Alexion Pharmaceuticals, Inc.; July 2024.
2. 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.
3. Jones SA, Plantaz D, Vara R, et al. Effect of sebelipase alfa on survival and liver function in infants with rapidly progressive lysosomal acid lipase deficiency. Molecular Gene Met. 2015;114 (2): S59.
4. Erwin AL. The role of sebelipase alfa in the treatment of lysosomal acid lipase deficiency. Therap Adv Gastroenterol. 2017 Jul; 10 (7): 553 – 62.
5. National Organization for Rare Disorders. Wolman disease. 2015. Available at: https://rarediseases.org/rare-diseases/wolman-disease/. Accessed on July 6, 2020.
6. National Organization for Rare Disorders. Cholesteryl ester storage disease. 2019. Available at: https://rarediseases.org/rare-diseases/cholesteryl-ester-storage-disease/. Accessed on July 6, 2020.
7. Jones SA, Rojas-Caro S, Quinn AG, et al. Survival in infants treated with sebelipase alfa for lysosomal acid lipase deficiency: an open-label, multicenter, dose-escalation study. Orphanet J Rare Dis. 2017 Feb 8; 12 (1): 25 – 36.
8. Balwani M, Breen C, Enns GM, et al, Clinical effect and safety profile of recombinant human lysosomal acid lipase in patients with cholesteryl ester storage disease. Hepatology. 2013 Sep; 58 (3): 950 – 7.
9. Valayannopoulos V, Malinova V, Honzík T, et al. Sebelipase alfa over 52 weeks reduces serum transaminases, liver volume and improves serum lipids in patients with lysosomal acid lipase deficiency. J Hepatol. 2014 Nov; 61 (5): 1135 – 42.
10. 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 Sep 10; 373 (11): 1010 - 20.

<table>
<tr><th colspan="3">Policy History</th></tr>
<tr><th>#</th><th>Date</th><th>Change Description</th></tr>
<tr><td>1.8</td><td>Effective Date: 10/03/2024</td><td>Annual review – no changes made to the criteria at this time</td></tr>
<tr><td>1.7</td><td>Effective Date: 10/12/2023</td><td>Updated to remove prescriber requirement</td></tr>
<tr><td>1.6</td><td>Effective Date: 10/06/2022</td><td>Annual review – no changes made to the criteria at this time</td></tr>
<tr><td>1.5</td><td>Effective Date: 10/07/2021</td><td>Annual review of policy. No changes were made to the criteria.</td></tr>
<tr><td>1.4</td><td>Effective Date: 10/08/2020</td><td>New policy created for this disease state and drug. The Enzyme Replacement Therapy policy will be retired</td></tr>
<tr><td>1.3</td><td>Effective Date: 02/01/2019</td><td>UM medical management system update for MAPPO
<table><tr><th>Line of Business</th><th>PA Required in Medical Management System (Yes/No)</th></tr><tr><td>BCBS</td><td>Yes</td></tr><tr><td>BCN</td><td>Yes</td></tr><tr><td>MAPPO</td><td>Yes</td></tr><tr><td>BCNA</td><td>Yes</td></tr></table></td></tr>
<tr><td>1.2</td><td>Effective Date: 01/01/2017</td><td>UM medical management system update for BCNA
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<tr><td>1.1</td><td>Effective Date: 02/01/2016</td><td>UM medical management system update for BCBS
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** The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or http://dailymed.nlm.nih.gov/dailymed/index.cfm.*