Kanuma® (sebelipase alfa) intravenous infusion 20mg/10mL

KANUMA® (sebelipase alfa) is an innovative enzyme replacement therapy.

How KANUMA Works

KANUMA (sebelipase alfa) works by replacing the deficient lysosomal acid lipase (LAL) enzyme and reducing the accumulation of fatty materials in the lysosomes of cells throughout the body. KANUMA is administered via intravenous infusion.[1]

Global Approvals

Approved in 40 countries around the world, including in the United States, the European Union, Japan and Canada for the treatment of lysosomal acid lipase deficiency (LAL-D)

Clinical Trials

For clinical trial and other information, visit Kanuma.com.

Please see U.S. Indication & Important Safety Information for KANUMA on page 2.

KANUMA is the first and only approved treatment to address the underlying cause of lysosomal acid lipase deficiency (LAL-D).

- LAL-D is a genetic, chronic and progressive ultra-rare metabolic disease.[2]
- LAL-D is caused by genetic mutations that result in a marked decrease or loss in activity of the lysosomal acid lipase (LAL) enzyme, which is responsible for the breakdown of lipid particles.[2] In patients with LAL-D, deficient LAL enzyme activity can lead to the continuous, accumulation of fatty material in vital organs, such as the liver, blood vessel walls and other tissues, potentially resulting in progressive, multi-organ damage.[2,3]
- In infants, LAL-D is a rapidly progressive disease that often results in liver failure and premature mortality within 12 months of age.[4,5] In children and adults, LAL-D progresses gradually and may lead to multi-organ damage, including liver fibrosis and cirrhosis, liver failure, an abnormal amount of lipids leading to accelerated atherosclerosis, cardiovascular disease and potentially premature death.[2,3]
- LAL-D affects people of all ages, and symptoms may manifest at any time from infancy through adulthood.[2,5]

MAUREEN & LAUREN
LIVING WITH LAL-D

ALEXION®

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Patient Access and Support

Alexion's objective is that every patient with LAL-D who can benefit from KANUMA will have access to it. As part of this commitment, Alexion's OneSource™ program offers a complimentary, personalized support program, designed to support the specific needs of all patients with conditions we serve.

Each LAL-D patient has a dedicated OneSource case manager who can help patients and their families understand their insurance benefits and provide information about reimbursement assistance.

Alexion also offers a co-pay assistance program for eligible patients with commercial insurance. Case managers can also share information on nonprofit independent foundations that provide both financial and treatment-related support for all patients, regardless of their type of insurance. For patients who cannot obtain access to KANUMA, the Alexion Access Foundation, a charitable foundation, provides KANUMA free of charge to qualifying patients in need.

Patients, caregivers, and healthcare providers in the United States can call 1.888.765.4747 or email OneSource@Alexion.com to speak with a OneSource case manager.

References

1. Kanuma (sebelipase alfa) injection. Prescribing Information. Alexion Pharmaceuticals, Inc. Initial U.S. Approval: 2015.
2. Bernstein DL, et al. Chloesterylester storage disease: review of the findings in 135 reported patients with an underdiagnosed disease. J Hepatol. 2013;58:1230-43. doi:10.1016/j.jhep.2013.02.014.
3. Reiner Z, et al. Lysosomal acid lipase deficiency – an under-recognized cause of dyslipidemia and liver dysfunction. Atherosclerosis. 2014;235:21-30. doi:10.1016/j.atherosclerosis.2014.04.003.
4. Jones SA, Valayannopoulos V, Schneider E, et al. Rapid progression and mortality of lysosomal acid lipase deficiency presenting in infants. Genet Med. 2016;18:452-458. doi: 10.1038/gim.2015.108.
5. Jones SA. Survival in infants treated with sebelipase alfa for lysosomal acid lipase deficiency: an open-label, multicenter, dose-escalation study. Orphanet Journal of Rare Diseases. 2017;12:25.

U.S. Indication & Important Safety Information for KANUMA® (sebelipase alfa)

INDICATION:

KANUMA (sebelipase alfa) is indicated for the treatment of patients with a diagnosis of lysosomal acid lipase deficiency (LAL-D).

IMPORTANT SAFETY INFORMATION:

Life-threatening and severe allergic reactions may occur in people who receive KANUMA. These reactions may occur in people who are starting treatment with KANUMA or in people who have previously received KANUMA without having an allergic reaction. Seek immediate medical care right away if you have any of the following signs or symptoms:

- Chest pain or discomfort
- Wheezing or trouble breathing
- Rash or hives
- Red eyes
- Swelling of eyelids
- Rapid heartbeat
- Rapid breathing
- Runny nose

Tell your doctor if you have had a severe allergic reaction to eggs or egg products, as people with a known history of egg allergies were excluded from clinical trials.

The most common side effects in patients treated with KANUMA are:

- In infants with rapidly progressive disease presenting within the first 6 months of life: diarrhea, vomiting, fever, stuffy or runny nose, low hemoglobin (red blood cells), cough, swelling of the nose and throat, and hives.
- In pediatric and adult patients: headache, fever, sore throat, swelling of the nose and throat, weakness, constipation, and nausea.

Tell your doctor if you are pregnant or plan to become pregnant, or are breastfeeding or plan to breastfeed.

These are not all of the possible side effects of KANUMA. Call your healthcare provider for medical advice about side effects. To report suspected side effects, contact Alexion at 1-844-259-6783 or the FDA at 1-800-FDA-1088.

Please see the full Prescribing Information for KANUMA®.

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