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EPAR summary for the public

Kanuma

sebelipase alfa

This is a summary of the European public assessment report (EPAR) for Kanuma. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Kanuma.

For practical information about using Kanuma, patients should read the package leaflet or contact their doctor or pharmacist.

What is Kanuma and what is it used for?

Kanuma is a medicine used to treat patients of all ages with lysosomal acid lipase deficiency. This is an inherited disease caused by the lack of an enzyme called lysosomal acid lipase, which is needed to break down fats within cells. When the enzyme is absent or present only in low levels, fats accumulate in the body's cells, causing symptoms such as growth failure and liver damage.

Because the number of patients with lysosomal acid lipase deficiency is low, the disease is considered 'rare', and Kanuma was designated an 'orphan medicine' (a medicine used in rare diseases) on 17 December 2010.

Kanuma contains the active substance sebelipase alfa.

How is Kanuma used?

Treatment with Kanuma should be supervised by a doctor experienced in the treatment of lysosomal acid lipase deficiency, other metabolic diseases or liver diseases. Treatment should be given by a trained healthcare professional who can manage medical emergencies (such as a severe allergy). The medicine can only be obtained with a prescription.

Kanuma is available as a concentrate to be made into a solution for infusion (drip) into a vein. The recommended dose is 1 mg per kilogram body weight given once every other week. The infusion should last around 1 to 2 hours.

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In patients who have rapidly progressing disease before 6 months of age, a dose of 1 mg/kg is given once a week instead of once every other week; in these patients the dose can be increased to 3 mg/kg once a week depending on the response to treatment.

Kanuma should be started as early as possible after diagnosis and is intended for long-term use.

How does Kanuma work?

The active substance in Kanuma, sebelipase alfa, is a copy of the enzyme that is lacking in patients with lysosomal acid lipase deficiency. Sebelipase alfa replaces the missing enzyme, helping to break down fats and stopping them building up in the body's cells.

What benefits of Kanuma have been shown in studies?

Kanuma has been studied in 2 main studies in patients with lysosomal acid lipase deficiency. The first study involved 9 infants with growth failure or other evidence of rapidly progressing disease in their first 6 months of life. The study showed that 6 out of the 9 infants given Kanuma survived to 1 year of age. Growth improvements were also observed in all 6 surviving infants.

The second study involved 66 patients (children and adults) and compared Kanuma with placebo (a dummy treatment). The main measure of effectiveness was the proportion of patients who achieved normal levels of a liver enzyme called ALT after 5 months of treatment. High levels of ALT enzymes are a sign of liver damage. In this study, 31% of the patients given Kanuma (11 out of 36) achieved normal levels of ALT enzymes, compared with 7% of the patients given placebo (2 out of 30).

What are the risks associated with Kanuma?

The most serious side effects with Kanuma (seen in around 3 patients in 100) are signs and symptoms of severe allergic reactions. These include chest discomfort, red eyes, eyelid swelling, difficulty breathing, itchy rash, hives, flushing, runny nose, rapid heartbeat and rapid breathing. Development of antibodies against the medicine has also been reported especially in infants. If antibodies develop, Kanuma may not work effectively. For the full list of all side effects reported with Kanuma, see the package leaflet.

Kanuma must not be used in patients who have had a life-threatening allergic reaction to the active substance which re-occurred after stopping and starting treatment again. It must also not be used in patients with life-threatening allergy to eggs or any of Kanuma's ingredients.

Why is Kanuma approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Kanuma's benefits are greater than its risks and recommended that it be approved for use in the EU. The Committee noted the lack of any effective treatments for lysosomal acid lipase deficiency and the high mortality in infants with rapidly progressing disease. The CHMP considered that Kanuma led to significant improvements in survival in infants, and was effective at improving symptoms of the disease in patients of all ages. Regarding safety, no major issues have been identified and serious side effects were rare or manageable. However, further data are needed on the long-term benefits and safety of the medicine.

What measures are being taken to ensure the safe and effective use of Kanuma?

A risk management plan has been developed to ensure that Kanuma is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Kanuma, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company that markets Kanuma is carrying out a study in infants with rapidly progressing disease and will set-up a registry of patients of all ages to obtain further information on the long-term benefits and safety of Kanuma, in particular on the risk of allergic reactions and the development of antibodies against the medicine. The company will also provide educational material to all doctors expected to prescribe Kanuma, encouraging them to enroll patients in the registry and informing them of how to monitor patients for antibodies and manage patients who develop severe allergic reactions.

Further information can be found in the summary of the risk management plan.

Other information about Kanuma

The European Commission granted a marketing authorisation valid throughout the European Union for Kanuma on 28 August 2015.

The full EPAR and risk management plan summary for Kanuma can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Kanuma, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Kanuma can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 08-2015.