



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

25 June 2015  
EMA/CHMP/425434/2015  
Press Office

## Press release

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# EMA fast-tracks enzyme replacement therapy for lysosomal acid lipase deficiency

## Kanuma is first treatment for rare life-threatening genetic disease causing multiple organ damage

The European Medicines Agency (EMA) has recommended granting a marketing authorisation for Kanuma (sebelipase alfa), for the treatment of lysosomal acid lipase (LAL) deficiency, a rare life-threatening inherited condition.

Patients with this condition have a wide range of symptoms such as growth failure, enlarged liver, diarrhoea and malabsorption (when nutrients from food are not easily absorbed during digestion). There are different types of LAL deficiency with the disease in infants, also called Wolman disease, being the most severe. In its most severe form, the disease is usually fatal in the first year of life.

There is currently no approved therapy for this condition. Due to the lack of treatment and the high death rate in infants under the age of one, EMA's Committee for Medicinal Products for Human Use (CHMP) decided to speed up the evaluation of this medicine and recommended marketing authorisation following an accelerated assessment. This mechanism is one of the Agency's tools to speed up patient access to new medicines where there is an unmet medical need.

LAL deficiency occurs when the body does not produce enough active lysosomal acid lipase (LAL), an enzyme which breaks down fatty material. The lack of the LAL enzyme can lead to a build-up of fatty material in a number of vital body organs including the liver and blood vessels.

Kanuma is indicated for long-term enzyme replacement therapy (ERT) in infants, children and adult patients. Kanuma's active substance is sebelipase alfa, a recombinant lysosomal human acid lipase, which is effective in replacing the activity of the missing enzyme. Kanuma is the first recombinant product produced from the egg white of transgenic hens (hens whose cells have been modified to include a foreign gene).

The CHMP based its recommendation on four studies which provided evidence on the safety and efficacy in infants (under six months of age), children and adults. LAL is a rare disease; therefore the number of participants in these studies was small. Across all four studies, a total of 106 patients with LAL deficiency received treatment with sebelipase alfa, including 14 infants. In the clinical trials significant improvements were observed for a number of disease parameters. There was an



improvement in survival of infants with LAL deficiency for which no treatment was available up until now. Collection of long-term efficacy data will continue through an ongoing study in infants with LAL deficiency.

The most common side effects with Kanuma were potential allergic reaction (hypersensitivity), high levels of fatty material in the blood (transient hyperlipidemia) and development of anti-drug antibodies (ADAs) which can hamper treatment as it might induce hypersensitivity and/or make treatment potentially less effective. The CHMP recommended that hypersensitivity, which appears to be more frequent in infants, should be further monitored following authorisation in all age groups. CHMP also required that long-term monitoring of ADAs in infants and in older populations be carried out.

Because LAL deficiency is rare, Kanuma was designated as an orphan medicine by the Committee for Orphan Medicinal Products (COMP). Orphan designation gives medicine developers access to incentives such as fee reductions for scientific advice and is the key instrument available in the EU to encourage the development of medicines for patients with rare diseases.

The opinion adopted by the CHMP at its June 2015 meeting is an intermediary step on Kanuma's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

## Notes

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1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Kanuma is Synageva BioPharma Ltd.
3. Following this positive CHMP opinion, the COMP will assess whether the orphan designation should be maintained.
4. More information on the work of the European Medicines Agency can be found on its website: [www.ema.europa.eu](http://www.ema.europa.eu)

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