25 June 2015
EMA/CHMP/359213/2015
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Kanuma

sebelipase alfa

On 25 June 2015, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Kanuma, intended for the treatment of lysosomal acid lipase (LAL) deficiency. Kanuma was designated as an orphan medicinal product on 17 December 2010. The applicant for this medicinal product is Synageva BioPharma Ltd.

Kanuma will be available as 2 mg/ml concentrate for solution for infusion. The active substance of Kanuma is sebelipase alfa, a recombinant human lysosomal acid lipase (rhLAL).

The benefits with Kanuma are its ability to replace the activity of the missing enzyme resulting in reduced liver fat content and reduced levels of blood transaminases, low-density lipoprotein (LDL) cholesterol, non-high-density lipoprotein (HDL) and triglycerides. In addition, there was a significant benefit in terms of survival (67%) in infants with Wolman disease beyond 12 months.

The most serious adverse reactions experienced by 3% of patients in clinical trials were signs and symptoms consistent with anaphylaxis. Signs and symptoms included chest discomfort, conjunctival injection, dyspnoea, generalised and itchy rash, hyperaemia, mild eyelid oedema, rhinorrhoea, severe respiratory distress, tachycardia, tachypnoea and urticaria.

The full indication is: "for long-term enzyme replacement therapy (ERT) in patients of all ages with lysosomal acid lipase (LAL) deficiency." It is proposed that Kanuma be prescribed by physicians experienced with the treatment of lysosomal acid lipase (LAL) deficiency or other metabolic disorders or chronic liver disease.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5520
Send a question via our website www.ema.europa.eu/contact