8 September 2015
EMA/COMP/470354/2015
Committee for Orphan Medicinal Products

# Recommendation for maintenance of orphan designation at the time of marketing authorisation

## Kanuma (sebelipase alfa) for the treatment of lysosomal acid lipase deficiency

During its meeting of 14 to 16 July 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/10/827 for Kanuma (sebelipase alfa, previously known as recombinant human lysosomal acid lipase) as an orphan medicinal product for the treatment of lysosomal acid lipase deficiency. The COMP assessed whether, at the time of marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the condition, and the existence of other methods of treatment. The COMP recommended that the orphan designation of the medicine be maintained[1].

### Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Kanuma for:

'long-term enzyme replacement therapy in patients of all ages with lysosomal acid lipase deficiency'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of lysosomal acid lipase deficiency'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2010. Lysosomal acid lipase deficiency remains a severe disease which, in its most severe form, is usually fatal in the first year of life.

### Prevalence of the condition

The sponsor provided updated information on the prevalence of lysosomal acid lipase deficiency based on data from the scientific literature.

[1] The maintenance of the orphan designation at time of marketing authorisation would, except in specific situations, give an orphan medicinal product 10 years of market exclusivity in the EU. This means that in the 10 years after its authorisation similar products with a comparable therapeutic indication cannot be placed on the market.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
**Telephone** +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
**Send a question via our website** www.ema.europa.eu/contact

An agency of the European Union

© European Medicines Agency, 2015. Reproduction is authorised provided the source is acknowledged.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of lysosomal acid lipase deficiency remains below the ceiling for orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was still estimated to be not more than 0.2 people in 10,000. This is equivalent to a total of not more than 10,000 people in the EU.

## Existence of other methods of treatment

The COMP noted that, at the time of the review of the orphan designation, no treatments were authorised in the EU for patients affected by this condition. Patients were advised to follow a diet low in fats. In some cases, haematopoietic (blood) stem cell transplantation had been used, but with modest results. This is a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow.

## Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Kanuma still meets the criteria for designation as an orphan medicinal product and that it should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Kanuma can be found in the European public assessment report (EPAR) on the Agency's website ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports.