10 June 2016
EMA/COMP/267525/2016
Committee for Orphan Medicinal Products

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

Galafold (migalastat) for the treatment of Fabry disease

On 8 April 2016, the Committee for Orphan Medicinal Products (COMP) completed its review of the designation EU/3/06/368 for Galafold (migalastat[1]) as an orphan medicinal product for the treatment of Fabry disease. 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. As other methods of treatment are authorised in the European Union (EU), the COMP also considered whether the medicine is of significant benefit to patients with Fabry disease. The COMP recommended that the orphan designation of the medicine be maintained[2].

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

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Galafold for the 'long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of Fabry disease'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2006. Fabry disease remains a condition that is debilitating in the long term particularly due to recurrent episodes of severe pain not responding to standard analgesics. The condition can also be life threatening because of kidney failure and complications affecting the heart and the nervous system.

[1] Previously known as 1-deoxygalactonojirimycin hydrochloride.
[2] 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 the same 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

Prevalence of the condition

The sponsor provided updated information on the prevalence of Fabry disease based on data from the published literature.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of Fabry disease 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 estimated to be less than 2.3 people in 10,000. This is equivalent to a total of fewer than 118,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, Fabrazyme and Replagal were authorised in the EU for the treatment of Fabry disease. These are two injectable medicines containing the enzyme α-galactosidase A, which is lacking in patients with Fabry disease.

Significant benefit of Galafold

The COMP concluded that the claim of a significant benefit of Galafold in the treatment of Fabry disease is justified because this medicine is available as capsules to be taken by mouth whereas existing treatment are given by infusion (drip) into a vein. This is considered a major contribution to patient care.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Galafold is of significant benefit to patients affected by Fabry disease.

Conclusions

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

Further information on the current regulatory status of Galafold 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.