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Committee for Orphan Medicinal Products

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

Procysbi (mercaptamine) for the treatment of cystinosis

During its meeting of 9-11 July 2013, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/10/778 for Procysbi (mercaptamine[1]) as an orphan medicinal product for the treatment of cystinosis. 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 satisfactory methods of treatment. As other methods of treatment for patients with this condition are authorised in the European Union (EU), the COMP also looked at the significant benefit of the product over existing treatments. 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 Procysbi for:

'treatment of proven nephropathic cystinosis. Cysteamine reduces cystine accumulation in some cells (e.g. leukocytes, muscle and liver cells) of nephropathic cystinosis patients and, when treatment is started early, it delays the development of renal failure'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2010. Cystinosis remains a condition that is long-term debilitating and life threatening, because it can lead to kidney failure if left untreated.

[1] Previously known as cysteamine bitartrate (gastroresistant).
[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 a comparable therapeutic indication cannot be placed on the market.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

Prevalence of the condition

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of cystinosis 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 approximately 0.1 people in 10,000. This is equivalent to a total of around 5,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, Cystagon (another mercaptamine-containing medicine) was authorised in the EU for the treatment of nephropathic cystinosis. This medicine is available as immediate-release capsules that need to be taken every six hours.

Significant benefit over existing treatments

The COMP noted that Procysbi is as effective as Cystagon in treating nephropathic cystinosis, and it has a similar safety profile. However, Procysbi is a new gastroresistant formulation of cysteamine that can be given twice a day (every 12 hours), while Cystagon has to be given four times a day (every six hours). The COMP concluded that the claim of a significant benefit of Procysbi over current treatment is justified on the basis that this more convenient formulation makes it easier for patients to stick to their treatment, interfering less with the patient's daily activities and their sleep. This is therefore considered to make 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 Procysbi is of significant benefit for patients affected by cystinosis.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Procysbi 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 Procysbi 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.