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Committee for Orphan Medicinal Products

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

Cystadrops (mercaptamine) for the treatment of cystinosis

During its meeting of 6 to 8 December 2016, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/08/578 for Cystadrops (mercaptamine, previously known as cysteamine hydrochloride) 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 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 cystinosis. 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 Cystadrops for:

'treatment of corneal cystine crystal deposits in adults and children from 2 years of age with cystinosis'.

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 2008. Cystinosis remains a debilitating and life-threatening condition because it progressively impairs kidney function and other organs, and can lead to sight loss and intolerance to light.

[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 the same therapeutic indication cannot be placed on the market.

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Prevalence of the condition

The sponsor provided updated information on the prevalence of cystinosis based on data from the scientific literature.

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 methods of treatment

At the time of the review of the orphan designation, Cystagon and Procysbi were authorised in the EU for treating nephropathic (kidney) cystinosis. These medicines are available as capsules containing mercaptamine and target the build-up of cystine crystals in the kidneys. No medicines were authorised for treating ocular (eye) cystinosis, but pharmacies and hospitals prepared their own mercaptamine eye drop solutions to decrease cystine crystals in the cornea.

Significant benefit of Cystadrops

The COMP concluded that the claim of a significant benefit of Cystadrops in cystinosis is justified because the medicine, which is available as eye drops, has been shown to be effective at reducing corneal cystine crystals in ocular cystinosis, for which no other treatments are currently authorised.

Therefore, although other methods for the treatment of cystinosis have been authorised in the EU, the COMP concluded that Cystadrops is of significant benefit to patients with cystinosis affecting the eye.

Conclusions

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

More information on the COMP assessment can be found in the December 2016 COMP minutes.

Further information on Cystadrops 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.