EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

26 October 2012
EMA/COMP/874717/2011
Committee for Orphan Medicinal Products

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

Bronchitol (mannitol) for the treatment of cystic fibrosis

During its meeting of 8-9 November 2011, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/05/325 for Bronchitol (mannitol, previously known as mannitolum) as an orphan medicinal product for the treatment of cystic fibrosis. 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 satisfactory 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[1].

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

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

'treatment of cystic fibrosis in adults aged 18 years and above as an add-on therapy to best standard of care'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2005. Cystic fibrosis remains a condition that is debilitating in the long term and life threatening, particularly due to respiratory insufficiency resulting from long-term lung infection.

Prevalence of the condition

The sponsor provided updated information on the prevalence of cystic fibrosis based on published literature and recent data from national registries.

[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.

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On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of cystic fibrosis 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 approximately 0.7 people in 10,000. This is equivalent to a total of around 35,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, other methods were authorised in the EU for the treatment of cystic fibrosis. To treat the lung disease component of cystic fibrosis, mucolytics such as rhDNase and acetylcysteine were used to dissolve the secretions and facilitate the clearance of bronchial mucus, leading to a reduction of lung infections and inflammation.

Significant benefit over existing treatments

The COMP noted that Bronchitol is an osmotic agent that works in a different way to authorised medicines to facilitate mucus clearance. It also noted that in a main study of cystic fibrosis patients, some of whom were taking rhDNase, Bronchitol showed a small but significant improvement in patients' forced expiratory volume in one second (FEV_1) as well as a modest reduction in exacerbations. The COMP concluded that the claim of significant benefit is justified on the basis of its mechanism of action and added efficacy when used in addition to currently used treatments such as rhDNase.

Therefore, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Bronchitol is of significant benefit for patients affected by cystic fibrosis.

Conclusions

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