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Committee for Orphan Medicinal Products

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

Tobi Podhaler (tobramycin) for the treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis

During its meeting of 6-7 October 2010, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/03/140 for Tobi Podhaler (tobramycin) as an orphan medicinal product for the treatment of *Pseudomonas aeruginosa* lung infection in 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 Tobi Podhaler for:

'the suppressive therapy of chronic pulmonary infection due to *Pseudomonas aeruginosa* in adults and children aged six years and older with cystic fibrosis'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of *P. aeruginosa* lung infection in cystic fibrosis'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2003. *P. aeruginosa* lung infection in cystic fibrosis remains a condition that is debilitating in the long term and life threatening, particularly because of the damage to the lung tissue that leads to progressive respiratory insufficiency.

[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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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 *P. aeruginosa* lung infection in cystic fibrosis remains below the threshold 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 1.3 people in 10,000. This is equivalent to a total of around 66,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, lung infections in patients with cystic fibrosis were mainly treated with antibiotics. These included tobramycin, an antibiotic that belongs to the group 'aminoglycosides'. Tobramycin was authorised as Tobi in several European Member States for the long-term treatment of *P. aeruginosa* infections in cystic fibrosis. Tobi is available as a solution for inhalation to be used with a nebuliser (a device that changes the solution into an aerosol that the patient can inhale).

Since the orphan designation of Tobi Podhaler, Cayston (aztreonam) has also received marketing authorisation in the EU for the suppressive therapy of chronic pulmonary infections due to *P. aeruginosa* in patients with cystic fibrosis aged 18 years and older. The active substance in Cayston, aztreonam, belongs to a different class of antibiotics, beta-lactams.

In addition, other medicines used to treat the lung symptoms of cystic fibrosis included bronchodilators (medicines that help open up the airways in the lungs) and mucolytics (medicines that help to dissolve the mucus). Patients were also advised to exercise and have physiotherapy.

Significant benefit over existing treatments

The COMP noted that Tobi Podhaler was at least as efficacious as Tobi in clinical trials in patients with cystic fibrosis aged six years and over. The COMP concluded that the claim of a significant benefit of Tobi Podhaler over Tobi is justified on the basis of the added convenience in the use of the medicine. Due to its formulation, Tobi Podhaler can be inhaled using a hand-held device, which allows a shorter inhalation time of 5.6 min compared with 19.7 min with Tobi. In addition, the improved portability of the device, which weighs 20 g and does not need electricity, is considered to be a major contribution to patient care.

When comparing Tobi Podhaler with Cayston, as well as with other antibiotics, the COMP noted that Tobi Podhaler has a different mechanism of action. The claim of significant benefit is justified on the basis of it being a complementary therapeutic option to treat *P. aeruginosa* infection in cystic fibrosis.

Therefore, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Tobi Podhaler is of significant benefit for patients affected by *P. aeruginosa* lung infection in cystic fibrosis.

Conclusions

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

Further information on the current regulatory status of Tobi Podhaler 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.