18 October 2011
EMA/CHMP/837842/2011
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Bronchitol

Mannitol

On 18 October 2011, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Bronchitol 40 mg inhalation powder, hard capsules intended for the treatment of cystic fibrosis (CF) in adults aged 18 years and above as an add-on therapy to best standard of care. Bronchitol was designated as an orphan medicinal product on 7 November 2005. The applicant for this medicinal product is Pharmaxis Pharmaceuticals Ltd.

The active substance of Bronchitol is mannitol, an hyperosmolar agent (R05CB16) which is understood to change the viscoelastic properties of mucus, increase the hydration of the periciliary fluid layer and contribute to increased mucociliary clearance of the retained secretions.

The benefits with Bronchitol are its ability to improve lung function. Although the size of the effect is small with around 2-3% absolute change in FEV_1 predicted and the clinical benefit is difficult to ascertain, it is acknowledged that even a small effect can be of relevance given the deterioration of FEV_1 inherent to the disease progression. The most commonly observed side effect is cough. The most important adverse reactions are bronchospasm and haemoptysis.

A pharmacovigilance plan for Bronchitol will be implemented as part of the marketing authorisation.

The approved indication is: "Bronchitol is indicated for the treatment of cystic fibrosis (CF) in adults aged 18 years and above as an add-on therapy to best standard of care." It is proposed that the patient's initiation dose must be used under the supervision and monitoring of an experienced physician or another health care professional appropriately trained and equipped to perform spirometry, monitor oxygen saturation (SpO_2), and manage acute bronchospasm including appropriate use of resuscitation equipment. Based on the available data, the safety and efficacy of Bronchitol in children and adolescents aged 6 to 18 years has not been established.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7<sector fax>
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers there to be a favourable benefit-to-risk balance for Bronchitol and therefore recommends the granting of the marketing authorisation.