

IV COMP DISCUSSION AND CONCLUSION AT DAY 90

Cystic fibrosis is a distinct medical entity and thus a valid condition. There exists scientific rationale for the development of Hypothiocyanite and lactoferrin for the treatment of the condition.

The sponsor has established that the condition was affecting approximately 1.3 in 10 000 persons in the Community when the application was made.

The sponsor has established that the condition is chronically debilitating and life threatening due to respiratory insufficiency which is a complication of chronic lung infection and premature death.

The sponsor has established that, despite existing authorised methods of treatment there remains an important need for improving existing treatments or improving the overall outcome of patients affected by the condition. Based on the pre-clinical evidence, the preliminary clinical data and the justifications provided, the assumption that Hypothiocyanite and lactoferrin will be of potential significant benefit for patients affected by the condition appears justified in particular with regards to clinical relevant advantage based on the new mechanism of action and its contribution to the overall treatment of patients affected by the condition.

The COMP recommends that protocol assistance is sought from the EMEA prior to submission of the application for marketing authorisation, particularly with regard to the data that will be required for the demonstration of significant benefit.

As other medicinal products are currently authorized for the treatment of the same condition, the sponsor's attention is drawn to the potential similarity between both medicinal products and the possible implications in the product development and requirements for marketing authorisation, according to Article 8 of Regulation (EC) No 141/2000. Further guidance on the application of Article 8 is available on the European Commission's web-site.

It should be highlighted that further to Article 5(12)(b) of Regulation (EC) No 141/2000 and Article B 2.1 of Communication from the Commission on Regulation (EC) No 141/2000, when a sponsor submits an application for marketing authorisation for a designated orphan medicinal product, it is the responsibility of the sponsor to submit a report on the criteria that led to the designation of the product as an orphan medicinal product and updated information on the current fulfillment of these criteria.

Due to the potential interest of developing the product in a paediatric indication in the proposed condition and in related conditions where the medicinal product could be used, the sponsor is advised to (i) consider the requirements of Regulation (EC) No 1901/2006 with regards to the development and application for marketing authorisation of the medicinal product, and (ii) consider the possibility to apply for orphan designation for those conditions affecting children and where a paediatric development can be requested.

V GROUNDS FOR THE OPINION ON ORPHAN MEDICINAL PRODUCT DESIGNATION

Whereas the Committee for Orphan Medicinal Products (COMP), having examined the application, concluded:

- cystic fibrosis (hereinafter referred to as "the condition") was estimated to be affecting approximately 1.3 in 10,000 persons in the Community, at the time the application was made;
- the condition is chronically debilitating and life threatening due to respiratory insufficiency which is a complication of chronic lung infection that may lead to premature death;
- although satisfactory methods of treatment of the condition have been authorised in the Community, sufficient justification has been provided that hypothiocyanite and lactoferrin may be of significant benefit to those affected by the condition.

The COMP recommends the designation of this medicinal product, containing hypothiocyanite and lactoferrin, as an orphan medicinal product for the orphan indication: treatment of cystic fibrosis.