European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2003 on request of the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF eflornithine hydrochloride for the treatment of Familial Adenomatous Polyposis (FAP)

On 19 February 2002, orphan designation (EU/3/02/087) was granted by the European Commission to ILEX Services Limited, United Kingdom, for eflornithine hydrochloride in the treatment of Familial Adenomatous Polyposis (FAP).

What is familial adenomatous polyposis?
Familial Adenomatous Polyposis (FAP), also known as familial polyposis coli, is a hereditary disease characterised by the appearance of numerous polyps throughout the large bowel. The average number of polyps in FAP patients is around 1,000, but this may vary between 100 and 2,500. FAP may lead to cancer of the large bowel and as such is a life-threatening condition.

What are the methods of treatment available?
At the time of submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for treatment of the condition. Available therapeutic methods consisted of standard screening, prophylactic colorectal surgery and endoscopic surveillance.

What is the estimated number of patients affected by the condition*?
According to the information provided by the sponsor, FAP was considered to affect about 11,300 to 37,600 patients in the European Union.

How is this medicinal product expected to act?
Eflornithine hydrochloride acts by blocking cell replication. Its mode of action has been attributed to the inhibition of an enzyme called ornithine decarboxylase.

What is the stage of development of this medicinal product?
The effects of eflornithine hydrochloride have been evaluated in experimental models. At the time of submission of the application for orphan designation, no clinical trials in patients with FAP had been initiated.

Eflornithine hydrochloride had not been marketed anywhere worldwide for FAP or designated as an orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 18 December 2001 a positive opinion recommending the grant of the above mentioned designation.

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Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to be affecting not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which have been considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.