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EMA/COMP/363907/2010
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Eflornithine for the treatment of familial adenomatous polyposis

On 20 September 2010, orphan designation (EU/3/10/779) was granted by the European Commission to Cancer Prevention Pharma Ltd, United Kingdom, for eflornithine for the treatment of familial adenomatous polyposis.

What is familial adenomatous polyposis?

Familial adenomatous polyposis (FAP) is a hereditary disease in which numerous polyps (growths) form in the gut, mainly in the large intestine. The polyps usually start to develop in late childhood and their number varies from hundreds to thousands. Patients with FAP may have blood in the stools, diarrhoea or constipation, abdominal pain (stomach ache) or weight loss with no obvious cause.

FAP is a long-term debilitating disease that may be life threatening because there is a high risk of it developing into cancer of the large intestine if it is not treated, and can cause problems outside the gut including stomach problems and other types of cancer.

What is the estimated number of patients affected by the condition?

At the time of designation, FAP affected approximately 0.2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 10,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, celecoxib was authorised in the EU for the treatment of FAP in addition to surgery (to remove the polyps) and endoscopic monitoring (to check if polyps are developing using an endoscope, a thin tube that allows a doctor to look inside the gut).

The sponsor has provided sufficient information to show that eflornithine might be of significant benefit for patients with FAP because early studies indicate that it might improve the treatment of patients

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 506,500,000 (Eurostat 2010).

with this condition. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Eflornithine works by blocking the action of an enzyme called ornithine decarboxylase, which is involved in stimulating cells to grow. In patients with FAP, polyps form because ornithine decarboxylase is overactivated. By blocking this enzyme, eflornithine is expected to slow down the formation of polyps. This medicine is to be available as tablets and as a powder to be taken by mouth.

What is the stage of development of this medicine?

The effects of eflornithine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with eflornithine in patients with FAP were planned.

At the time of submission, eflornithine cream was authorised for the treatment of facial hirsutism in women (excessive growth of coarse hair on the face) in the EU as well as in several countries worldwide.

At the time of submission, eflornithine was not authorised anywhere in the EU for FAP. Orphan designation of eflornithine had been previously granted for FAP in the EU, but this designation had been later withdrawn by the sponsor.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 2 June 2010 recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

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

For more information

Sponsor's contact details:

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For contact details of patients' organisations whose activities are targeted at rare diseases see:

- [Orphanet](#), a database containing information on rare diseases which includes a directory of patients' organisations registered in Europe.
- [European Organisation for Rare Diseases \(EURORDIS\)](#), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Eflornithine	Treatment of familial adenomatous polyposis
Bulgarian	Ефлорнитин	Лечение на Фамилна Аденоматозна Полипоза
Czech	Eflornithin	Léčba familiární adenomatózní polypózy
Danish	Eflornithin	Behandling af familiær adenomatøs polypose
Dutch	Eflornithine	Behandeling van familiale adenomateuze polyposis
Estonian	Eflornitiin	Perekondliku adenomatoosse polüpoosi ravi
Finnish	Eflornitiini	Perinnöllisen adenomatoottisen polyypypitaudin hoito
French	Eflornithine	Traitement de la polypose adénomateuse familiale
German	Eflornithin	Behandlung von familiärer adenomatöser Polyposis
Greek	Εφλορνιθίνη	Θεραπεία της οικογενούς αδενωματοδούς πολυποδίασης
Hungarian	Eflornitin	Familiáris adenomatosus polyposis kezelése
Italian	Eflornitina	Trattamento della poliposi familiare adenomatosa
Latvian	Eflornitīns	Ģimenes adenomatozās polipozes ārstēšana
Lithuanian	Eflornitinas	Šeiminės adenomatozinės polipozės gydymas
Maltese	Eflornithine	Kura tal-polipoži adenomatuża li tintiret
Polish	Eflornityna	Leczenie Rodzinnej Polipowatości Gruczolakowatej
Portuguese	Eflornitina	Tratamento da polipose adenomatosa familiar
Romanian	Eflornitină	Tratamentul polipozei adenomatoase familiale
Slovak	Eflornitín	Liečba familiárnej adenomatóznej polypózy
Slovenian	Eflornitin	Zdravljenje familiarne adenomatozne polipoze
Spanish	Eflornitina	Tratamiento de la poliposis colónica familiar
Swedish	Eflornitin	Behandling av familjär adenomatös polypos
Norwegian	Eflornitin	Behandling av familiær adenomatøs polypose
Icelandic	Eflornithín	Ættlægt adenomatös sepager

¹ At the time of designation