



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

London, 13 October 2009  
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## **Committee for Orphan Medicinal Products**

### **Public summary of positive opinion for orphan designation of eicosapentaenoic acid for the treatment of familial adenomatous polyposis**

On 8 October 2009, orphan designation (EU/3/09/666) was granted by the European Commission to S.L.A. Pharma (UK) Ltd, United Kingdom, for eicosapentaenoic acid for the treatment of familial adenomatous polyposis.

#### **What is familial adenomatous polyposis?**

Familial adenomatous polyposis (FAP) is a hereditary disease characterised by the formation of numerous polyps (growths), mainly in the large intestine. 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) and weight loss for no obvious reason.

FAP is a long-term debilitating disease that may be life threatening because of the high risk of developing into cancer of the large intestine if it is not treated.

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

At the time of designation, FAP affected less than 1 in 10,000 people in the European Union (EU) \*. This is equivalent to a total of fewer than 50,000 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 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 eicosapentaenoic acid might be of significant benefit for patients with FAP because early studies indicate that it might be effective in reducing the number of polyps and might have fewer side effects than existing treatments. 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?**

Eicosapentaenoic acid is an omega-3 fatty acid that is obtained from fish oil. The way that eicosapentaenoic acid works in FAP is not fully understood, but increasing the intake of eicosapentaenoic acid has been shown to change the composition of fats in the cell membrane. In particular, it is thought that eicosapentaenoic acid replaces another fat in the cell membrane called

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\*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 504,800,000 (Eurostat 2009).

arachidonic acid, which is involved in the inflammation process. Replacing arachidonic acid with eicosapentaenoic acid is expected to reduce inflammation. This may help to suppress the formation of polyps in the intestine.

**What is the stage of development of this medicine?**

The effects of eicosapentaenoic acid have been evaluated in experimental models.

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

At the time of submission, eicosapentaenoic acid was not authorised anywhere in the EU for FAP or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2009 recommending the granting of this designation.

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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 Community) 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:**

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

<b>Language</b>	<b>Active ingredient</b>	<b>Indication</b>
English	Eicosapentaenoic acid	Treatment of familial adenomatous polyposis
Bulgarian	Ейкозапентаенова киселина	Лечение на Фамилна Аденоматозна Полипоза
Czech	Kyselina eikosapentaenová	Léčba familiární adenomatózní polypózy
Danish	Eicosapentaensyre	Behandling af familiær adenomatøs polypose
Dutch	Eicosapentaenoiczuur	Behandeling van familiale adenomateuze polyposis
Estonian	Eikosapentaanhape	Perekondliku adenomatoosse polüpoosi ravi
Finnish	Eikosapentaeenihappo	Perinnöllisen adenomatoottisen polyypitaudin hoito
French	Acide eicosapentanoïque	Traitement de la polypose adénomateuse familiale
German	Eicosapentaensäure	Behandlung von familiärer adenomatöser Polyposis
Greek	Εικοσιπεντανικό οξύ	Θεραπεία της οικογενούς αδενωματοδούς πολύποσης
Hungarian	Eikozapentén-sav	Familiáris adenomatosus polyposis kezelése
Italian	Acido eicosapentaenoico	Trattamento della poliposi familiare adenomatosa
Latvian	Eikozapentaenoiskā skābe	Ģimenes adenomatozās polipozes ārstēšana
Lithuanian	Eikozapentanoinė rūgštis	Šeiminės adenomatozinės polipozės gydymas
Maltese	Eicosapentaenoic acid	Kura tal-polipozi adenomatuża li tintiret
Polish	Kwas eikozapentaenowy	Leczenie Rodzinnej Polipowatości Guczkolakowatej
Portuguese	Ácido eicosapentaenóico	Tratamento do pólipo adenomatoso familiar
Romanian	Acid eicosapentaenoic	Tratamentul polipozei adenomatoase familiale
Slovak	Kyselina eikozapentaénová	Liečba familiárnej adenomatóznej polypózy
Slovenian	Eikozapentaenojska kislina	Zdravljenje familiarne adenomatozne polipoze
Spanish	Ácido eicosapentaenoico	Tratamiento de la poliposis colónica familiar
Swedish	Ikosapentanoisk syra	Behandling av familjär adenomatös polypos
Norwegian	Eikosapentaensyre	Behandling av familiær adenomatøs polypose
Icelandic	Eikósapentensýra	Ættlægt adenomatös sepager