



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

Document Date: London, 15 June 2009
Doc.Ref.: EMEA/COMP/264/04

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of celecoxib for the treatment of Familial Adenomatous Polyposis (FAP)

On 20 November 2001, orphan designation (EU/3/01/070) was granted by the European Commission to Pharmacia-Pfizer EEIG, United Kingdom, for celecoxib for the treatment of familial adenomatous polyposis (FAP).

The sponsorship was transferred to Pfizer Limited, United Kingdom, in December 2007.

What is familial adenomatous polyposis?

Familial Adenomatous Polyposis (FAP), also known as familial polyposis coli, is a hereditary disease characterised by the progressive appearance of numerous polyps mainly in the large intestine. Polyps develop as early as in childhood. The average number of polyps in FAP patients is around 1,000, but this may vary between 100 and 2,500. Polyps are initially benign but can easily become cancerous. FAP may lead to cancer of the large intestine, and as such is a life-threatening condition.

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

At the time of designation FAP affected approximately 0.3 to 1 in 10,000 people in the European Union (EU) *. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 11,000-38,000 people.

What treatments are 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 endoscopic surveillance with removal of polyps when required. Prophylactic surgery to remove a part of the large intestine is performed if the polyps are numerous or the polyps are becoming cancerous. The patients also receive genetic counselling as the disease is inherited.

How is this medicine expected to work?

Celecoxib is an anti-inflammatory medicine. Its mode of action has been attributed to the inhibition of prostaglandin synthesis, via inhibition of an enzyme (protein molecules that act as catalysts in the cells biochemical reactions) called cyclooxygenase-2 (COX-2). Prostaglandins are a class of hormone-like (chemical messenger) lipids (fats) present in tissues and bodily fluids. They are involved in processes such as pain, inflammation and kidney function. In FAP, celecoxib is thought to induce cell death, and thus prevent or delay the growth of polyps.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union. This represents a population of 377,000,000 (Eurostat 2001).

What is the stage of development of this medicine?

The effects of celecoxib were evaluated in experimental models.

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

At the time of submission of the application for orphan designation, celecoxib was authorised for symptomatic relief in the treatment of osteoarthritis or rheumatoid arthritis in Sweden. Celecoxib was not authorised anywhere worldwide for FAP, at the time of submission. Orphan designation of celecoxib was granted in Australia in April 2000 for FAP.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 7 September 2001 a positive opinion recommending the grant of the above mentioned designation.

Update: celecoxib (Onsenal) has been authorised in the EU since 17 October 2003 for the reduction of the number of adenomatous intestinal polyps in familial adenomatous polyposis (FAP), as an adjunct to surgery and further endoscopic surveillance.

The effect of Onsenal-induced reduction of polyp burden on the risk of intestinal cancer has not been demonstrated.

For more information: www.emea.europa.eu/htms/human/epar/a.htm

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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information:

Sponsor's contact details:

Pfizer Limited,

Ramsgate Road,

Sandwich,

Kent CT13 9NJ,

United Kingdom

Telephone: +44 13 04 64 85 30

Telefax: +44 13 04 65 50 47

E-mail: jim.barras@pfizer.com

Patients' associations contact points:

Ligue Nationale Contre le Cancer

14 Rue Corvisart

75013 Paris

France

Telephone: +33 1 53 55 24 00

Telefax: +33 1 43 36 91 10

E-mail: ligue@ligue-cancer.net

Macmillan Cancer Support (merged with CancerBACUP)

3 Bath Place

Rivington Street

London

EC2A 3JR

United Kingdom

Telephone: +44 20 7696 9003

Switchboard open during office hours, Mon–Fri, 9am–Noon and 2pm–4.45pm

Telefax: +44 20 7696 9002

Deutsche Krebshilfe e.V.

Buschstr. 32

53113 Bonn

Germany

Telephone: +49 2 287 29 900

Telefax: +49 2 287 29 90 11

E-mail: deutsche@krebshilfe.de

Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Celecoxib	Treatment of Familial Adenomatous Polyposis
Bulgarian	Целекоксиб	Лечение на Фамилна Аденоматозна Полипоза (ФАП)
Czech	Celecoxib	Léčba familiární adenomatózní polypózy (FAP)
Danish	Celecoxib	Behandling af familiær adenomatøs polyposis
Dutch	Celecoxib	Behandeling van familiale adenomateuze polyposis
Estonian	Tselekoksiib	Perekondliku adenomatoosse polüpoosi ravi
Finnish	Selekoksibi	Familiaalisen adenomatoottisen polypoosin hoito
French	Celecoxib	Traitement de la polypose familiale adénomateuse
German	Celecoxib	Behandlung der familiären adenomatösen Polyposis
Greek	Celecoxib	Θεραπεία της οικογενής αδενωματώδους πολυποδίασης.
Hungarian	Celecoxib	Familiáris adenomatosus polyposis kezelése
Italian	Celecoxib	Trattamento della Poliposi Adenomatosa Familiare
Latvian	Celekoksibs	Ģimenes adenomatozās polipozes ārstēšana
Lithuanian	Celekoksibas	Šeiminės adenomatozinės polipozės (ŠAP) gydymas
Maltese	Celecoxib	Kura tal-polipoži adenomatuża li tintiret (FAP)
Polish	Celecoxib	Leczenie Rodzinnej Polipowatości Gruczolakowatej
Portuguese	Celecoxib	Tratamento da Polipose Adenomatosa Familiar
Romanian	Celecoxib	Tratamentul polipozei adenomatoase familiale
Slovak	Celecoxib	Liečba familiárnej adenomatóznej polypózy (FAP)
Slovenian	Celekoksib	Zdravljenje farmiliarne adenomatozne polipoze (FAP)
Spanish	Celecoxib	Tratamiento de Poliposis Adenomatosa Familiar
Swedish	Celecoxib	Behandling av familjär adenomatös kolonpolypos