



EUROPEAN MEDICINES AGENCY
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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Valproic acid, sodium for the treatment of familial adenomatous polyposis

First publication	4 January 2006
Rev.1: withdrawal from the Community Register	21 November 2012
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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in November 2012 on request of the sponsor.

On 30 November 2004, orphan designation (EU/3/04/246) was granted by the European Commission to G2M Cancer Drugs AG, Germany for valproic acid, sodium for the treatment of familial adenomatous polyposis.

What is familial adenomatous polyposis?

Familial adenomatous polyposis (FAP), also known as familial polyposis coli, is an inherited disease characterised by the appearance of numerous masses called "polyps" throughout the large bowel. Other abnormalities involving other organs such as the eyes, the bones or the skin can also be present. The disease is caused by a defect (called mutation) in the gene that normally inhibits (blocks) the formation of the polyps. Patients with FAP usually have to undergo removal of a big part of their bowel, which is chronically debilitating. FAP may also lead to cancer of the large bowel and as such is a life-threatening condition.



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

At the time of designation, familial adenomatous polyposis affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 23,000 people*, and is below the ceiling 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 are the methods of treatment available?

Available non-pharmacological methods for the treatment of the familial adenomatous polyposis consist of surgical removal of the large bowel followed by regular visual inspections of the bowel cavities using a flexible instrument called endoscope. Medicinal treatments such as anti-inflammatory agents are authorised and used for the condition in the Community at the time of submission of the application for orphan designation.

Valproic acid, sodium might be of potential significant benefit for the treatment of familial adenomatous polyposis because it may act in a different way than other available treatments. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Valproic acid, sodium is expected to stop (inhibit) the formation of the new polyps and it might also reduce the size of the existing polyps. Although it is not yet completely understood how it works, it is suggested by the sponsor that it might act on a certain substance normally produced by the body cells, the so called histone deacetylase, that is involved in the generation of the polyps.

What is the stage of development of this medicine?

The effects of valproic acid, sodium were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with familial adenomatous polyposis were initiated.

Valproic acid, sodium was not marketed anywhere worldwide for the treatment of familial adenomatous polyposis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2004 recommending the granting of this designation.

*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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 464,200,000 (Eurostat 2004).

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	Valproic acid, sodium	Treatment of familial adenomatous polyposis
Czech	Kyselina valproová, natrium	Léčba familiární adenomatosní polyposy
Danish	Valproesyre, natrium	Behandling af familiær adenomatøs polypose
Dutch	Valproïnezuur, natrium	Behandeling van familiale adenomateuze polyposis
Estonian	Valproaathape, naatrium	Perekondliku adenomatoosse polüpoosi ravi
Finnish	Valproaattihappo, natrium	Perinnöllisen adenomatoottisen polyypypitaudin hoito
French	Acide Valproïque, sodium	Traitement de la polypose adénomateuse familiale
German	Valproinsäure, Natrium	Behandlung von familiärer adenomatöser Polyposis
Greek	βαλπροϊκό οξύ, άλας νατρίου	Θεραπεία της οικογενούς αδενωματοδους πολύποσης
Hungarian	Valproát sav, nátrium	Familiáris adenomatosus polyposis kezelése
Italian	Acido valproico, sale sodico	Trattamento della poliposi familiare adenomatosa
Latvian	Valproiskābe nātrija sāls	Ģimenes adenomatozās polipozes ārstēšana
Lithuanian	Valproinė rūgštis, natris	Šeiminės adenominės polipozės gydymas
Maltese	Valproic acid, sodium	Treatment of familial adenomatous polyposis
Polish	Walproinian sodu	Leczenie gruczolakowatej polipowatości rodzinnej
Portuguese	Ácido valpróico, sal sódico	Tratamento do pólipó adenomatoso familiar
Slovak	Valproát sodný	Liečba familiárnej adenomatóznej polypózy
Slovenian	Natrijev valproat	Zdravljenje familiarne adenomatozne polipoze
Spanish	Ácido valproico, sal sódica	Tratamiento de la poliposis colónica familiar
Swedish	Natriumvalproat	Behandling av familjär adenomatös polypos
Norwegian	Natriumvalproat	Behandling av familiær adenomatøs polypose
Icelandic	Valpróin syra	Ættlægt adenomatös sepager

¹ At the time of designation