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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Glufosfamide for the treatment of pancreatic cancer

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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.	

On 15 April 2011, orphan designation (EU/3/11/851) was granted by the European Commission to Theradex (Europe) Ltd, United Kingdom, for glufosfamide for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: to produce a juice that helps with the digestion of food, and to produce hormones such as insulin. Due to the absence of symptoms in the early stages of pancreatic cancer, the majority of patients are diagnosed when the cancer has spread locally or to other parts of the body.

Pancreatic cancer is a very severe and life-threatening disease that is associated with shortened life expectancy.

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

At the time of designation, pancreatic cancer affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 66,000 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).

*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. At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).



What treatments are available?

At the time of designation, several medicines were authorised in the EU for treating pancreatic cancer. The choice of treatment depended on several factors, including how advanced the disease is. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that glufosfamide might be of significant benefit for patients with pancreatic cancer because of its new mechanism of action and the results of early studies which show that it might improve the treatment of patients 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?

Glufosfamide is made of another anticancer medicine called isophosphoramide mustard linked to glucose (a sugar). It is a cytotoxic (cell-killing) substance that belongs to the group 'alkylating agents'. Alkylating agents kill cancer cells by attaching to their DNA while they are reproducing. As a result, cancer cells cannot reproduce and this slows down the growth of tumours. The glucose in the medicine is expected to help glufosfamide enter the tumour cells.

What is the stage of development of this medicine?

The effects of glufosfamide have been evaluated in experimental models.

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

At the time of submission, glufosfamide was not authorised anywhere in the EU for pancreatic cancer. Orphan designation had been granted in the United States of America for pancreatic cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 January 2011 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	Glufosfamide	Treatment of pancreatic cancer
Bulgarian	Глуфосфамид	Лечение на рак на панкреаса
Czech	Glufosfamide	Léčba karcinomu pankreatu
Danish	Glufosfamid	Behandling af pancreascancer
Dutch	Glufosfamide	Behandeling van pancreaskanker
Estonian	Glufosfamiid	Pankreasevähi ravi
Finnish	Glufosfamidi	Haimasyövän hoito
French	Glufosfamide	Traitement du cancer pancréatique
German	Glufosfamid	Behandlung des Pankreaskarzinoms
Greek	Γλουφωσφαμίδη	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Glufoszfamid	Hasnyálmirigyrák kezelése
Italian	Glufosfamide	Trattamento del cancro pancreatico
Latvian	Glufosfamīds	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Glufosfamidaz	Kasos vėžio gydymas
Maltese	Glufosfamide	Kura tal-kanċer tal-frixa
Polish	Glufosfamid	Leczenie raka trzustki
Portuguese	Glufosfamida	Tratamento do carcinoma do pâncreas
Romanian	Glufosfamidă	Tratamentul cancerului pancreatic
Slovak	Glufosfamid	Liečba rakoviny pankreasu
Slovenian	Glufosfamid	Zdravljenje raka trebušne slinavke
Spanish	Glufosfamida	Tratamiento del cáncer de páncreas
Swedish	Glufosfamid	Behandling av pankreascancer
Norwegian	Glufosamid	Behandling av pankreascancer
Icelandic	Glúfosfamíð	Meðferð bráskrabbameins

¹ At the time of designation