



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

5'-O-(*trans*-9''-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine for the treatment of pancreatic cancer

First publication	16 November 2009
Rev.1: transfer of sponsorship	17 May 2011
Rev.2: withdrawal from the Community Register	11 June 2013
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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2013 on request of the Sponsor.

On 28 October 2009, orphan designation (EU/3/09/680) was granted by the European Commission to Clavis Pharma ASA, Norway, for 5'-O-(*trans*-9''-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine (CP-4126) for the treatment of pancreatic cancer.

The sponsorship was transferred to Gregory Fryer Associates Ltd, United Kingdom, in June 2010 and subsequently to Clovis Oncology UK Limited, United Kingdom, in March 2011.

What is pancreatic cancer?

Pancreatic cancer is cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: producing a juice that helps with the digestion of food, and producing hormones such as insulin. Due to the absence of symptoms in the early stages of the disease, 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 leads to poor long-term survival.



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 is equivalent to a total of around 66,000 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 submission of the application for orphan drug designation, several medicines were authorised for pancreatic cancer in the EU. The choice of treatment for pancreatic cancer 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 5'-O-(*trans*-9''-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine might be of significant benefit for patients with pancreatic cancer because early studies in experimental models indicate that it might improve the treatment of patients with this condition, in particular a sub-group of patients who do not respond to 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?

5'-O-(*trans*-9''-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine belongs to the group 'anti-metabolites'. In the body, this medicine is expected to be incorporated into the genetic material of cells (DNA and RNA) and interfere with the enzymes involved in making new DNA and RNA. As a result, it is expected to inhibit the growth of tumour cells and eventually kill them.

What is the stage of development of this medicine?

The effects of 5'-O-(*trans*-9''-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine have been evaluated in experimental models.

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

At the time of submission, 5'-O-(*trans*-9''-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine was not authorised anywhere in the EU for pancreatic cancer or designated as 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 2 September 2009 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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 504,800,000 (Eurostat 2009).

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:

Clovis Oncology UK Ltd
Sheraton House
Castle Park
Castle Hill
Cambridge CB3 0AX
United Kingdom
Telephone: +44 1223 370 037
E-mail: info@clovisoncology.com

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	5'-O-(trans-9''-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine	Treatment of pancreatic cancer
Bulgarian	5'-O-(транс-9''-октадеценоил-1-β-D-2'-2'деокси-2',2'-дифлуороцитидин	Лечение на рак на панкреаса
Czech	5'-O-(trans-9''-oktadecenoyl)-1-β-D-2'deoxy-2',2'-difluorcytidin	Léčba karcinomu pankreatu
Danish	5'-O-(trans-9''-octadecenoyl)-1-β-D-2'deoxy-2',2'-difluorcytidin	Behandling af pancreascancer
Dutch	5'-O-(trans-9''-octadecenoyl)-1-β-D-2'-desoxy-2',2'-difluorcytidine	Behandeling van pancreaskanker
Estonian	5'-O-(trans-9''-oktadetsenoüül)-1-β-D-2'deoksü-2',2'-difluorotsütidiin	Pankreasevähi ravi
Finnish	5'-O-(trans-9''-oktadekenoyyli)-1-β-D-2'deoksi-2',2'-difluorisytidiini	Haimasyövän hoito
French	5'-O-(trans-9''-octadéceoyl)-1-β-D-2'-deoxy-2',2'-difluorocytidine	Traitement du cancer pancréatique
German	5'-O-(trans-9''-Octadecenoyl)-1-β-D-2'deoxy-2',2'-difluorcytidin	Behandlung des Pankreaskarzinoms
Greek	5'-O-(trans-9''-δεκαοκτενοϋλ)-1-β-D-2'-δεσοξυ-2',2'-διφθοροκυτιδίνη	Θεραπεία καρκίνου του παγκρέατος
Hungarian	5'-O-(transz-9''-oktadecenoil)-1-β-D-2'dezoxi-2',2'-difluorcitidin	Hasnyálmirigyrák kezelése
Italian	5'-O-(trans-9''-ottadecenoil)-1-β-D-2'deossi-2',2'-difluorocitidina	Trattamento del cancro pancreatico
Latvian	5'-O-(trans-9''-oktadekenoil)-1-β-D-2'deoksi-2',2'-difluorocitidīns	Aizkuņģa dziedzerā vēža ārstēšana
Lithuanian	5'-O-(trans-9''-oktadecenoil)-1-β-D-2'deoksi-2',2'-difluorocitidinas	Kasos vėžio gydymas
Maltese	5'-O-(trans-9''-octadecenoyl)-1-beta-D-2'-deoxy-2',2'-difluorocytidine	Kura tal-kanċer tal-frixa
Polish	5'-O-(trans-9''-oktadecenoilo)-1-β-D-2'-deoksy-2',2'-difluorocytydyna	Leczenie raka trzustki
Portuguese	5'-O-(trans-9''-octadecenoil)-1-β-D-2'-desoxy-2',2'-difluorocitidina	Tratamento do carcinoma do pâncreas
Romanian	5'-O-(trans-9''-octadecenoil)-1-β-D-2'deoxi-2',2'-difluorcitidină	Tratamentul cancerului pancreatic
Slovak	5'-O-(trans-9''-oktadecenoyl)-1-β-D-2'deoxy-2',2'-difluórcytidín	Liečba rakoviny pankreasu
Slovenian	5'-O-(trans-9''-oktadecenoil)-1-β-D-2'deoksi-2',2'- difluorocitidin	Zdravljenje raka trebušne slinavke
Spanish	5'-O-(trans-9''-octadecenoil)-1-β-D-2'desoxi-2',2'-difluorocitidina	Tratamiento del cáncer de páncreas

¹ At the time of designation

Language	Active ingredient	Indication
Swedish	5'-O-(trans-9''-oktadecenoyl)-1-beta-D-2'-deoxi-2',2'-difluorocytidin	Behandling av pankreascancer
Norwegian	5'-O-(<i>trans</i> -9''-oktadekenoyl)-1-beta-D-2'-deoksy-2',2'-difluorcytidin	Behandling av pankreascancer
Icelandic	5'-O-(<i>trans</i> -9''-oktadekenóyl)-1-β-D-2'deoxý-2',2'-díflúrósýtidín	Meðferð briskrabbameins