



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Brivudine for the treatment of pancreatic cancer

On 28 January 2010, orphan designation (EU/3/09/703) was granted by the European Commission to RESprotect GmbH, Germany, for brivudine 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 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 is associated with 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 brivudine might be of significant benefit for patients with pancreatic cancer because early studies indicate that, when used in combination with existing treatments, it might improve the overall outcome of patients with this condition. This

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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).



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?**

Brivudine is expected to work by blocking the activity of a protein called 'heat shock protein 27 (Hsp27)', which is found in high amounts in pancreatic cancer cells. Hsp27 is known to play a key role in 'chemoresistance'. This means that it makes cancer cells not respond ('resistant') to chemotherapy, helping them to survive. When brivudine is given together with other anticancer medicines, it is expected to prevent the cancers cells from developing resistance to the medicines, helping to treat the disease.

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

The effects of brivudine have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 November 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**

Sponsor's contact details:

RESprotect GmbH  
Fiedlerstr. 34  
01307 Dresden  
Germany  
Telephone: +49 351 4503201  
Telefax: +49 51 4503210  
E-mail: [fahrig@resprotect.de](mailto:fahrig@resprotect.de)

## **Patient 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](mailto:ligue@ligue-cancer.net)

### **Macmillan Cancer Support**

3 Bath Place  
Rivington Street  
London EC2A 3JR  
United Kingdom  
Telephone: +44 20 7696 9003  
Telefax: +44 20 7696 9002

### **European Cancer Patient Coalition**

ECPC Office  
Am Rothenanger 1b  
85521 Riemerling  
Germany  
Telephone: +49 89 628 36 807  
Telefax: +49 89 628 36 808  
E-mail: [info@ecpc-online.org](mailto:info@ecpc-online.org)

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

Language	Active ingredient	Indication
English	Brivudine	Treatment of pancreatic cancer
Bulgarian	Бривудин	Лечение на рак на панкреаса
Czech	Brivudin	Léčba karcinomu pankreatu
Danish	Brivudin	Behandling af pancreascancer
Dutch	Brivudine	Behandeling van pancreaskanker
Estonian	Brivudiin	Pankreasevähi ravi
Finnish	Brivudiini	Haimasyövän hoito
French	Brivudine	Traitement du cancer pancréatique
German	Brivudin	Behandlung des Pankreaskarzinoms
Greek	Μπριβουδίνη	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Brivudin	Hasnyálmirigyrák kezelése
Italian	Brivudina	Trattamento del cancro pancreatico
Latvian	Brivudīns	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Brivudinas	Kasos vėžio gydymas
Maltese	Brivudine	Kura tal-kanċer tal-frixa
Polish	Brivudyna	Leczenie raka trzustki
Portuguese	Brivudina	Tratamento do carcinoma do pâncreas
Romanian	Brivudină	Tratamentul cancerului pancreatic
Slovak	Brivudin	Liečba rakoviny pankreasu
Slovenian	Brivudin	Zdravljenje raka trebušne slinavke
Spanish	Brivudina	Tratamiento del cáncer de páncreas
Swedish	Brivudin	Behandling av pancreascancer
Norwegian	Brivudin	Behandling av pancreascancer
Icelandic	Brívúdíń	Meðferð briskrabbameins