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## **Committee for Orphan Medicinal Products**

### **Public summary of positive opinion for orphan designation of paclitaxel (liposomal) for the treatment of pancreatic cancer**

On 31 October 2006, orphan designation (EU/3/06/419) was granted by the European Commission to MediGene AG, Germany, for paclitaxel (liposomal) for the treatment of pancreatic cancer.

#### **What is pancreatic cancer?**

The pancreas is a small organ that is located in the abdomen, behind the stomach. The pancreas has two main functions in the body; it produces a mix of enzymes (proteins that trigger chemical reactions) that help digest (break down) food and it produces hormones, such as insulin, which help to control blood sugar levels. Cancer of the pancreas is often detected at a late stage of the disease, because it rarely causes any symptoms in its earlier stages. Pancreatic cancer is life-threatening.

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

At the time of designation pancreatic cancer affected approximately 1.2 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of around 55,000 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?**

The choice of the treatment of pancreatic cancer depends on several factors, including the stage of the disease. Treatment may include surgery, radiation therapy (using high-dose x-rays or other high-energy rays to kill cancer cells), and chemotherapy (using drugs to kill cancer cells). Several medicinal products have been authorised for the treatment of pancreatic cancer in the Community. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that paclitaxel (liposomal) might be of potential significant benefit for the treatment of pancreatic cancer, because it may improve the long-term outcome of the patients. 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?**

When cells divide and grow, there are structures (tubules) inside the cells that need to assemble and disassemble in a very orderly way. Paclitaxel interferes with the assembly of these tubules and subsequently with the growth of cells. Liposomal paclitaxel is delivered to the cancer cells in the tumour in little lipid particles (liposomes) that bind specifically to the cells that line blood vessels. According to the sponsor, paclitaxel (liposomal) will, by inhibiting the growth of newly formed blood vessels, contribute to the destruction of the tumour.

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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 459,700,000 (Eurostat 2004).

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

The effects of paclitaxel (liposomal) were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with pancreatic cancer were ongoing.

Paclitaxel (liposomal) was not authorised anywhere worldwide for the treatments of pancreatic cancer nor designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 4 October 2006 a positive opinion recommending the grant of the above-mentioned 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.

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

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
English	Paclitaxel (liposomal)	Treatment of pancreatic cancer
Czech	Paclitaxel (liposomální)	Léčba karcinomu pankreatu
Danish	Paclitaxel (liposomal)	Behandling af pancreascancer
Dutch	Paclitaxel (liposomal)	Behandeling van pancreaskanker
Estonian	Paclitaxel (liposomaalne)	Pankreasevähi ravi
Finnish	Paklitakseeli (liposomaalinen)	Haimasyövän hoito
French	Paclitaxel (liposomal)	Traitement du cancer pancréatique
German	Paclitaxel (liposomal)	Behandlung des Pankreaskarzinoms
Greek	Paclitaxel (λιποσωμική)	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Paclitaxel (liposzómában)	Hasnyálmirigyrák kezelése
Italian	Paclitaxel (liposomale)	Trattamento del cancro pancreatico
Latvian	Paclitaxel (liposomu)	Aizkuņģa dziedzera vēža ārstēšanai
Lithuanian	Paklitakselis (liposomų)	Kasos vėžio gydymas
Polish	Paklitaksel(postać liposomalna)	Leczenie raka trzustki
Portuguese	Paclitaxel (lipossomal)	Tratamento do cancro do pâncreas
Slovak	Paklitaxel (lipozomálny)	Liečba rakoviny pankreasu
Slovenian	Paclitaxel (liposomalni)	Zdravljenje raka trebušne slinavke
Spanish	Paclitaxel (liposomal)	Tratamiento del cáncer de páncreas
Swedish	Paclitaxel (liposomal)	Behandling av pankreascancer
Norwegian	Paklitakselis (liposomalt)	Behandling av pankreascancer
Icelandic	Paclitaxel (í fitukornum)	Meðferð briskrabbameins