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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Nanoparticle albumin-bound paclitaxel for the treatment of pancreatic cancer

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2013 on request of the Sponsor.

On 26 November 2010, orphan designation (EU/3/10/809) was granted by the European Commission to Abraxis BioScience Limited, United Kingdom, for nanoparticle albumin-bound paclitaxel for the treatment of pancreatic cancer.

The sponsorship was transferred to Celgene Europe Limited, United Kingdom, in August 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: 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 pancreatic cancer?

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 threshold 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 treatments are available?

At the time of designation, several medicines were authorised for treating pancreatic cancer in the EU. The choice of treatment depended on several factors, including how advanced the disease is.

*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 506,300,000 (Eurostat 2010).

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Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

Nanoparticle albumin-bound paclitaxel is already authorised in the EU for the treatment of breast cancer under the name 'Abraxane'. The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with pancreatic cancer, as early studies show improved outcomes in patients when it is used in combination with other 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?

Paclitaxel, the active substance in nanoparticle albumin-bound paclitaxel, belongs to the group of anticancer medicines known as the 'taxanes'. Paclitaxel blocks the ability of cancer cells to break down their internal 'skeleton' that allows them to divide and multiply. With their skeleton still in place, the cells cannot divide and they eventually die.

Paclitaxel has been available as an anticancer medicine since 1993 but is not authorised for pancreatic cancer. Conventional types of paclitaxel contain substances that dissolve the paclitaxel, but which can cause hypersensitivity (allergic) reactions. Nanoparticle albumin-bound paclitaxel does not contain these substances. Instead, the paclitaxel is attached to a human protein called albumin in tiny particles known as 'nanoparticles'. This makes it easy to prepare a suspension of paclitaxel, which can be infused into a vein. The nanoparticles may also modify the way the medicine is distributed within the body, and this is expected to have a positive effect on its benefits and risks, in comparison with conventional medicines containing paclitaxel.

What is the stage of development of this medicine?

The effects of nanoparticle albumin-bound paclitaxel have been evaluated in experimental models.

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

At the time of submission, nanoparticle albumin-bound paclitaxel was authorised under the name 'Abraxane' for the treatment of breast cancer in the EU and in several countries outside the EU.

At the time of submission, nanoparticle albumin-bound paclitaxel was not authorised anywhere in the EU for pancreatic cancer. Orphan designation of the medicine had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 September 2010 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 European Union) 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Nanoparticle albumin-bound paclitaxel	Treatment of pancreatic cancer
Bulgarian	Паклитаксел, свързан с албумин в наночастици	Лечение на рак на панкреаса
Czech	Paklitaxel ve formě nanočástic vázaný na albumin	Léčba karcinomu pankreatu
Danish	Albuminbundet nanopartikel paclitaxel	Behandling af pancreascancer
Dutch	Nanopartikel albumine-gebonden paclitaxel	Behandeling van pancreaskanker
Estonian	Albumiiniga seotud paklitakseel nanoosakestena	Pankreasevähi ravi
Finnish	Paklitakselin albumiiniin sidottu nanopartikkeli	Haimasyövän hoito
French	Nanoparticules de paclitaxel lié à l'albumine	Traitement du cancer pancréatique
German	Albumingebundene Nanopartikel-Formulierung von Paclitaxel	Behandlung des Pankreaskarzinoms
Greek	Νανοσωματίδιο συνδεδεμένης με αλβουμίνη πακλιταξέλης	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Albuminhoz kötött paklitaxel nanorészecske	Hasnyálmirigyrák kezelése
Italian	Paclitaxel legato all'albumina in nanoparticelle	Trattamento del cancro pancreatico
Latvian	Nanodaļiņu albumīna saistītais paklitaksels	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Su albuminu sujungtas nanodalelių paklitakselis	Kasos vėžio gydymas
Maltese	Nanopartiċella ta' paclitaxel imwaħħal mal-albumina	Kura tal-kanċer tal-frixa
Polish	Nanocząstki paklitakselu związanego z albuminą	Leczenie raka trzustki
Portuguese	Nanopartículas de Paclitaxel ligadas à albumina	Tratamento do carcinoma do pâncreas
Romanian	Paclitaxel legat de albumină sub formă de nanoparticule	Tratamentul cancerului pancreatic
Slovak	Nanočastice na albumín viazaného paklitaxelu	Liečba rakoviny pankreasu
Slovenian	Paklitaksel, vezan na albuminske nanodelce	Zdravljenje raka trebušne slinavke
Spanish	Nanoparticulas de Paclitaxel vinculadas a albumina	Tratamiento del cáncer de páncreas
Swedish	Albuminbunden nanopartikelformulering av paklitaxel	Behandling av pankreascancer
Norwegian	Albuminbundet nanopartikkelformulering av paklitaksel	Behandling av pankreascancer
Icelandic	Öreindaralbúmín-bundið paclitaxel	Meðferð briskrabbameins

[1] At the time of designation