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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Trabedersen for the treatment of pancreatic cancer

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Rev.1: sponsor's name and address change	21 March 2014
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 24 July 2009, orphan designation (EU/3/09/660) was granted by the European Commission to Antisense Pharma GmbH, Germany, for trabedersen for the treatment of pancreatic cancer.

In February 2014, Antisense Pharma GmbH changed name to Isarna Therapeutics GmbH.

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. About 95% of pancreatic cancers affect the cells that make the pancreatic juice. These are called adenocarcinomas.

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 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 61,000 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 504,800,000 (Eurostat 2009).



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

The sponsor has provided sufficient information to show that trabedersen might be of significant benefit for patients with pancreatic cancer. It works in a different way to existing treatments and early studies indicate that trabedersen might be an alternative to existing treatments. These assumptions 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?

Trabedersen is an 'antisense oligonucleotide', a short piece of DNA that has been designed to attach to the genetic material of cells responsible for producing a protein called TGF- β 2. This blocks the production of TGF- β 2. TGF- β 2 is produced in large quantities in pancreatic cancer cells, and is involved in the growth, progression, and spreading of the cancer, as well as in the suppression of the body's immune system (the body's natural defences). By blocking the production of TGF- β 2, trabedersen is expected to stop the cancer cells from growing and multiplying. In addition, blocking the production of TGF- β 2 may stimulate the immune system to attack the cancer cells.

What is the stage of development of this medicine?

The effects of trabedersen 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, trabedersen 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 4 June 2009 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	Trabedersen	Treatment of pancreatic cancer
Bulgarian	Трабедерсен	Лечение на рак на панкреаса
Czech	Trabedersen	Léčba karcinomu pankreatu
Danish	Trabedersen	Behandling af pancreascancer
Dutch	Trabedersen	Behandeling van pancreaskanker
Estonian	Trabedersen	Pankreasevähi ravi
Finnish	Trabederseeni	Haimasyövän hoito
French	Trabédersen	Traitement du cancer pancréatique
German	Trabedersen	Behandlung des Pankreaskarzinoms
Greek	Τραμπεντερσέν	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Trabedersen	Hasnyálmirigyrák kezelése
Italian	Trabedersen	Trattamento del cancro pancreatico
Latvian	Trabederzēns	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Trabedersenas	Kasos vėžio gydymas
Maltese	Trabedersen	Kura tal-kanċer tal-frixa
Polish	Trabedersen	Leczenie raka trzustki
Portuguese	Trabedersen	Tratamento do carcinoma do pâncreas
Romanian	Trabedersen	Tratamentul cancerului pancreatic
Slovak	Trabedersen	Liečba rakoviny pankreasu
Slovenian	Trabedersen	Zdravljenje raka trebušne slinavke
Spanish	Trabedersén	Tratamiento del cáncer de páncreas
Swedish	Trabedersen	Behandling av pankreascancer
Norwegian	Trabedersen	Behandling av pankreascancer
Icelandic	Trabedersen	Meðferð briskrabbameins

¹ At the time of designation