12 March 2015
EMA/COMP/177332/2008 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human telomerase reverse transcriptase peptide (611-626) for the treatment of pancreatic cancer

First publication	1 April 2009
Rev.1: transfer of sponsorship	2 September 2009
Rev.2: sponsor's change of address	12 March 2015
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 25 July 2006, orphan designation (EU/3/06/384) was granted by the European Commission to Pharmexa A/S, Denmark, for human telomerase reverse transcriptase peptide (611-626) for the treatment of pancreatic cancer.

The sponsorship was transferred to Gemvax A/S, Norway, in May 2009.

What is pancreatic cancer?

Pancreatic cancer is a 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.

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

At the time of designation, pancreatic cancer affected approximately1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 56,000 people*, and is below the ceiling

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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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 submission of the application for orphan drug designation, several medicines were authorised for the treatment of pancreatic cancer in the European Union. The choice of treatment for pancreatic cancer depends on several factors, including the stage of the disease. Treatments may include surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines used to treat cancer). Satisfactory argumentation has been submitted by the sponsor to justify the assumption that human telomerase reverse transcriptase peptide (611-626) might be of potential significant benefit for the treatment of pancreatic cancer, mainly because it has a new mechanism of action and may be used in combination with other treatments. 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?

Human telomerase reverse transcriptase peptide (611-626) is a part of the enzyme (a protein that triggers chemical reactions) telomerase reverse transcriptase, which is often present in pancreatic cancer tumour cells. Human telomerase reverse transcriptase is needed for tumour cells to be able to divide many times (proliferate) and subsequently for the tumour to grow. The medicinal product is designed to activate the body's natural defence system, the immune system against the cells containing the human telomerase reverse transcriptase. According to the sponsor the product will trigger the immune system against the pancreatic cancer tumour cells, thus destroying the tumour cells with the body's own defence system.

What is the stage of development of this medicine?

The effects of human telomerase reverse transcriptase peptide (611-626) 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.

Human telomerase reverse transcriptase peptide (611-626) was not authorised anywhere worldwide for the treatment of pancreatic cancer nor designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 June 2006 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:

Gemvax A/S
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0273 Oslo
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Tel. + 47 22 44 75 73

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	Human telomerase reverse transcriptase peptide (611-626)	Treatment of pancreatic cancer
Bulgarian	Човешки теломераза обратна транскриптаза пептид (611-626)	Лечение на рак на панкреаса
Czech	Lidský telomerázový peptid s reverzní transkriptázou (611-626)	Léčba karcinomu pankreatu
Danish	Human telomerase omvendt transkriptase peptid (611-626)	Behandling af pancreascancer
Dutch	Humaan telomerase reverse transcriptase peptide (611-626)	Behandeling van pancreaskanker
Estonian	Inimese telomeraasi pöördtranskriptaasi peptiid (611-626)	Pankreasevähi ravi
Finnish	Ihmisen telomeraasin käänteistranskriptaasin peptidi (611-626)	Haimasyövän hoito
French	Peptide de la transcriptase inverse de la télomérase humaine (611-626)	Traitement du cancer pancréatique
German	Humane Telomerase Reverse Transkriptase-Peptid (611-626)	Behandlung des Pankreaskarzinoms
Greek	Πεπτίδιο ανθρώπινης τελομεράσης ανάστροφης τρανσκριπτάσης (611-626)	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Humán telomeráz reverz transzkriptáz peptid (611-626)	Hasnyálmirigyrák kezelése
Italian	Peptide della trascrittasi inversa della telomerasi umana (611-626)	Trattamento del cancro pancreatico
Latvian	Cilvēka telomerāzes reversās transkriptāzes peptīds (611-626)	Aizkuņģa dziedzera vēža ārstēšanai
Lithuanian	Žmogaus telomerazės atvirkštinės transkriptazės baltymas (611-626)	Kasos vėžio gydymas
Maltese	Telomerase reverse transcriptase peptide (611-626) uman	Kura tal-kanċer tal-frixa
Polish	Peptyd (611-626) ludzkiej telomerazy odwrotnej transkryptazy (hTERT)	Leczenie raka trzustki
Portuguese	Péptido da transcriptase reversa da telomerase humana (611-626)	Tratamento do cancro do pâncreas
Romanian	Telomeraz-revers transcriptaza (peptida 611-626) umană	Tratamentul cancerului pancreatic
Slovak	Peptid ľudskej telomerázovej reverznej transkriptázy (611-626)	Liečba rakoviny pankreasu
Slovenian	Peptid človeške telomerazne reverzne transkriptaze (611-626)	Zdravljenje raka trebušne slinavke

[1] At the time of designation

Language	Active Ingredient	Indication
Spanish	Péptido (611-626) de la transcriptasa inversa de la telomerasa humana	Tratamiento del cáncer de páncreas
Swedish	Mänsklig telomeras omvänt transkriptaspeptid (611-626)	Behandling av pankreascancer
Norwegian	Human telomerase omvendt transkriptase peptid (611-626)	Behandling av pankreascancer
Icelandic	Telómerasa-bakritapeptíð úr mönnum (611-626)	Meðferð briskrabbameins