15 August 2011
EMA/COMP/404700/2011
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mixture of seven synthetic fragments consisting of p21 RAS peptides for the treatment of pancreatic cancer

On 5 August 2011, orphan designation (EU/3/11/885) was granted by the European Commission to Targovax AS, Norway, for a mixture of seven synthetic fragments consisting of p21 RAS peptides for 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 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 the condition?

At the time of designation, pancreatic cancer affected approximately 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 51,000 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).

What treatments are available?

At the time of designation, several medicines were authorised in the EU for treating pancreatic cancer. The choice of treatment depended on several factors, including how far the disease had advanced. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

*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 506,300,000 (Eurostat 2011).

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The sponsor has provided sufficient information to show that the mixture of seven synthetic fragments consisting of p21 RAS peptides might be of significant benefit for patients with pancreatic cancer because of its new mechanism of action and the results of early studies, which show that it might improve the treatment of patients with this condition. 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?

The medicine contains seven synthetic peptides (short chains of amino acids) that are also found in a protein called 'p21 RAS'. The peptides contain mutations which are normally only expressed by certain cancer cells such as pancreatic cancer cells. The medicine is expected to act as a vaccine by 'teaching' the specialised cells of the body's immune system called T cells (a type of white blood cell) to recognise the p21 RAS proteins containing the mutations. It is expected that this will lead the T cells to attack and kill the pancreatic cancer cells containing these proteins.

What is the stage of development of this medicine?

The effects of the mixture of seven synthetic fragments consisting of p21 RAS peptides have been evaluated in experimental models.

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

At the time of submission, the medicinal product was not authorised anywhere in the EU for pancreatic cancer or designated as an 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 5 May 2011 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Mixture of seven synthetic fragments consisting of p21 RAS peptides	Treatment of pancreatic cancer
Bulgarian	Смес от 7 синтетични фрагмента, състоящи p21 RAS пептиди	Лечение на рак на панкреаса
Czech	Směs sedmi syntetických fragmentů sestávajících z p21 RAS peptidů	Léčba karcinomu pankreatu
Danish	Blanding af 7 syntetiske peptider bestående af p21 RAS peptider	Behandling af pancreascancer
Dutch	Mengsel van zeven synthetische fragmenten samengesteld uit p21Ras peptiden	Behandeling van pancreaskanker
Estonian	Segu seitsmest sünteetilisest fragmendist, mis sisaldavad P21 RAS peptiide	Pankreasevähi ravi
Finnish	p21 RAS peptideista koostuvien 7 synteettisen fragmenttien sekoitus	Haimasyövän hoito
French	Combinaison de 7 peptides synthétiques contenant des fragments de peptides p21 Ras	Traitement du cancer pancréatique
German	Kombination von sieben synthetischen p21 ras Peptid-Fragmenten	Behandlung des Pankreaskarzinoms
Greek	Μείγμα 7 συνθετικών θραυσμάτων που αποτελούν τμήματα πεπτιδίων p21 RAS	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Hét, p21 RAS peptidekből álló szintetikus fragmentum keveréke	Hasnyálmirigyrák kezelése
Italian	Combinazione di 7 frammenti sintetici costituiti da peptidi di RAS p21	Trattamento del cancro pancreatico
Latvian	7 sintētisko p21 RAS peptīdu fragmentu kombinācija,	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Septynių sintetinių fragmentų, sudarytų iš p21 RAS peptidų, mišinys	Kasos vėžio gydymas
Maltese	Taħlita ta' 7 frammenti sintetiċi li jikkonsistu minn peptidi RAS p21	Kura tal-kanċer tal-frixa
Polish	Mieszanina siedmiu syntetycznych fragmentów składających się z peptydów RAS p21	Leczenie raka trzustki
Portuguese	Combinação de 7 de peptídeos sintéticos constituídos por fragmentos de peptídeos p21 RAS	Tratamento do carcinoma do pâncreas
Romanian	Amestec de 7 fragmente peptidice de sinteză compuse din peptide p21 RAS	Tratamentul cancerului pancreatic
Slovak	Zmes siedmich syntetických fragmentov s obsahom p21 RAS peptidov	Liečba rakoviny pankreasu
Slovenian	Kombinacija 7 sintetičnih fgragmentov p21 peptidov RAS	Zdravljenje raka trebušne slinavke
Spanish	Combinación de 7 de fragmentos sintéticos de péptidos p21 RAS	Tratamiento del cáncer de páncreas

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Blanding av 7 syntetiska peptider bestående av fragment av p21 RAS-peptider	Behandling av pankreascancer
Norwegian	Blanding av sju syntetiske fragmenter bestående av p21 RAS peptider	Behandling av pankreascancer
Icelandic	Sambland af 7 syntetískum peptíðum sem samanstendur af brotum úr P21 RAS peptíðum	Meðferð briskrabbameins