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EMA/COMP/847393/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Two allogeneic irradiated pancreatic tumour cell lines for the treatment of pancreatic cancer

On 11 January 2016, orphan designation (EU/3/15/1604) was granted by the European Commission to Medpace Germany GmbH, Germany, for two allogeneic irradiated pancreatic tumour cell lines (also known as GVAX) for the 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 fluid 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 nearby 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 less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 103,000 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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with pancreatic cancer because early results suggest that it may improve survival of patients whose disease had not responded to previous therapy, when given as part of a combination treatment. 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 works by stimulating the patient's immune system, the body's natural defences, so that it targets and destroys the cancer cells. It is made of two types of pancreatic cancer cells that have been modified to produce granulocyte-macrophage colony stimulating factor (GM-CSF) and have been treated with radiation to prevent them from growing. GM-CSF stimulates the immune system to recognise as 'foreign' certain proteins, such as mesothelin, which are found at high levels on the surface of pancreatic cancer cells. This is expected to stimulate the immune system to destroy pancreatic cancer cells.

The medicine is given as part of a combination treatment including another medicine (called 'live attenuated *Listeria monocytogenes* delta *actA*/delta *inlB* strain expressing human mesothelin' or CRS-207) which helps the immune system to recognise mesothelin as foreign and so stimulate the body to attack the cancer.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 December 2015 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Two allogenic irradiated pancreatic tumour cell lines	Treatment of pancreatic cancer
Bulgarian	Две алогенни клетъчни линии от облъчени панкреатични туморни клетки	Лечение на рак на панкреаса
Croatian	Dvije linije alogenih ozračenih stanica tumora pankreasa	Liječenje raka gušterače
Czech	Dvě alogenní ozářené buněčné linie karcinomu pankreatu	Léčba karcinomu pankreatu
Danish	To allogene bestrålede cellelinjer fra pancreastumor	Behandling af pancreascancer
Dutch	Twee allogene bestraalde pancreastumorcellijnen	Behandeling van pancreaskanker
Estonian	Kaks allogeenset kiiritatud pankrease tuumori rakuliini	Pankreasevähi ravi
Finnish	Kaksi allogeenista säteilytettyä haiman kasvainsolulinjaa	Haimasyövän hoito
French	Deux lignées cellulaires tumorales pancréatiques allogéniques irradiées	Traitement du cancer pancréatique
German	Zwei allogene bestrahlte Pankreaskarzinom-Zelllinien	Behandlung des Pankreaskarzinoms
Greek	Δύο αλλογενείς ακτινοβοληµένες παγκρεατικές καρκινικές κυτταρικές σειρές	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Két allogén, besugárzott hasnyálmirigytumor sejtvonal	Hasnyálmirigyrák kezelése
Italian	Due linee cellulari tumorali pancreatiche allogeniche irradiate	Trattamento del cancro pancreatico
Latvian	Divas alogēnas apstarotas aizkuņģa dziedzera audzēja šūnu līnijas	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Dvi alogeninės, paveiktos spinduliuote kasos naviko ląstelių linijos	Kasos vėžio gydymas
Maltese	Żewġ linji ta' ċelloli ta' tumur tal-frixa alloġeniċi rradjati	Kura tal-kanċer tal-frixa
Polish	Dwie allogeniczne napromieniowane linie komórkowe guza trzustki	Leczenie raka trzustki
Portuguese	Duas linhas de células tumorais pancreáticas alogénicas irradiadas	Tratamento do carcinoma do pâncreas
Romanian	Două linii de celule tumorale pancreatice alogene iradiate	Tratamentul cancerului pancreatic
Slovak	Dve alogénne ožiarené pankreatické nádorové bunkové línie	Liečba rakoviny pankreasu
Slovenian	Dve alogenski obsevani celični liniji tumorja trebušne slinavke	Zdravljenje raka trebušne slinavke
Spanish	Dos líneas celulares tumorales pancreáticas alogénicas irradiadas	Tratamiento del cáncer de páncreas

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Två allogena bestrålade bukspottkörteltumörcellslinjer	Behandling av pankreascancer
Norwegian	To allogene bestrålte cellelinjer fra bukspyttkjerteltumor	Behandling av pankreascancer
Icelandic	Tvær ósamgena geislaðar frumulínur úr brisæxli	Meðferð briskrabbameins