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EMA/COMP/625102/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine *alpha-(1,3)-galactosyltransferase* gene for the treatment of pancreatic cancer

On 10 October 2012, orphan designation (EU/3/12/1048) was granted by the European Commission to European Medical Advisory Services Limited, United Kingdom, for mixture of two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine *alpha-(1,3)-galactosyltransferase* gene (also known as algenpantucel-L) 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 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 1.3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 66,000 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.

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

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with pancreatic cancer because early studies show that it works in a different way to existing treatments and might improve the outcome of patients with this condition when used together with currently authorised 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?

The medicine is expected to work as a 'cancer vaccine', by activating the patient's immune system (the body's natural defences) so that it attacks and kills the cancer cells. The medicine is made of pancreatic cancer cells that have been modified to produce an enzyme called alpha-(1,3)-galactosyltransferase. This enzyme is responsible for the production of substances called alpha-Gal, which are known to trigger an immune response. When the patient is given the vaccine, the patient's immune system is expected to stimulate an immune response not only against the cancer cells in the vaccine which contain alpha-Gal, but also against the cancer cells in the patients even though they do not contain alpha-Gal.

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, this medicine 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 5 September 2012 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 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 two allogeneic human pancreatic cancer cell lines stably transduced with a retroviral vector encoding the murine *alpha-(1,3)-galactosyltransferase* gene	Treatment of pancreatic cancer
Bulgarian	Смес от две алогенни човешки клетъчни линии от клетки на панкреатичен карцином, стабилно трансдуцирани с ретровирусен вектор, кодиращ миши ген на *алфа-(1,3)-галактозилтрансфераза*	Лечение на рак на панкреаса
Czech	Směs dvou allogenních humánních buněčných linií karcinomu pankreatu trvale transdukovaných retrovirálním vektorem kódujícím myší gen *alfa -(1,3) galactosyltransferázy*	Léčba karcinomu pankreatu
Danish	Blanding af to allogene humane pankreascancer cellelinier der er transduceret med en retroviral vektor som koder for det murine *alfa-(1,3) galactosyltransferase* gen	Behandling af pancreascancer
Dutch	Mengsel van twee allogene humane pancreaskankercellijnen die stabiel getransduceerd werden met een retrovirale vector, dewelke voor het murine *alpha-(1,3)-galactosyltransferase* gen codeert	Behandeling van pancreaskanker
Estonian	Segu kahest allogeensest inimese pankreaserakuliinist, mis on stabiilselt transdutseeritud retroviirusvektoriga, mis kodeerib hiire *alfa -(1,3)-galaktosüültransferaasi geeni*	Pankreasevähi ravi
Finnish	Sekoitus kahdesta ihmisen allogeenisesta haimasyöpäsolulinjasta, jotka on stabiilisti transdusoitu retrovirusvektorilla, joka enkoodaa hiiren *alfa-(1,3)-galaktosyylitransferaasigeeniä*	Haimasyövän hoito
French	Mélange de 2 lignées cellulaires humaines de cáncer pancréatique génétiquement modifiées par un vecteur retroviral codant pour le gène murin de *l'alpha-(1,3)-galactosyltransférase*	Traitement du cancer pancréatique
German	Kombination zweier allogener humaner Pankreaskarzinom-Zelllinien, die mit einem retroviralen Vektor transduziert wurden, welcher für das murine *alpha-(1,3)-Galactosyltransferase* Gen	Behandlung des Pankreaskarzinoms

[1] At the time of designation

Language	Active ingredient	Indication
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Greek	Μείγμα δύο αλλογενών ανθρώπινων κυτταρικών σειρών καρκίνου του πακρέατος σταθερά επιμολυσμένων με ένα ρετροϊικό φορέα που κωδικοποιεί το γονίδιο του ποντικού για την *α (1,3)-γαλακτοσυλτρανσφεράση*	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Két, rágcsáló *alfa-(1,3)-galaktoziltranszferáz* gént kódoló retrovirális vektorral stabilan transzdukált allogén humán hasnyálmirigyrák sejtvonal keveréke	Hasnyálmirigyrák kezelése
Italian	Mistura contenente due linee cellulari allogeneiche pancreatiche umane geneticamnet modificate con un vettore retrovirale codificante per il gene murino dell' *alfa (1,3) galactosiltransferasi*	Trattamento del cancro pancreatico
Latvian	Divu allogēnu cilvēka aizkuņģa dziedzera vēža šūnu līniju sajaukums, kurš noturīgi veidots ar retrovīrusa vektoru iekodētu peles *alfa-(1,3)-galaktoziltransferāzes* gēnā	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Dviejų alogeninių žmogaus kasos vėžio ląstelių linijų, stabiliai pakeistų retrovirusiniu vektoriumi, kuris koduoja pelės *alfa-(1,3)-galaktoziltransferazės* geną, mišinys	Kasos vėžio gydymas
Maltese	Taħlita ta' żewġ linji ta' ċelluli tal-kanċer tal-frixa umani alloġeniċi trasformati b'mod stabbli ma' vettur retrovirali li jikkodifika il-ġene tal-ġrieden tal-*alpha-(1,3)-galactosyltransferase*	Kura tal-kanċer tal-frixa
Polish	Mieszanina dwu allogenicznych ludzkich linii komórkowych raka trzustki stabilnie transfekowanych wektorem retrowirusowym kodującym gen *alfa-(1,3)-galaktozylotransferazy* mysiej	Leczenie raka trzustki
Portuguese	Mistura de duas linhas celulares humanas alogénicas de cancro do pancreas estavelmente transduzidas com um vector retroviral que codifica o gene murino *alfa-(1,3)-galactosiltransferase*	Tratamento do carcinoma do pâncreas
Romanian	Amestec de 2 linii celulare alogenice umane de cancer pancreatic genetic supuse unei transducţii stabile cu un vector retroviral codant pentru gena murină a *alfa-(1, 3)-galactoziltransferazei*	Tratamentul cancerului pancreatic
Slovak	Zmes dvoch línií alogénnych ľudských buniek rakoviny pankreasu stabilne transdukovaných s retrovírusovým vektorom kódujúcim myší gén pre *alfa-(1,3)-galaktozyltransferázu*	Liečba rakoviny pankreasu

Language	Active ingredient	Indication
Slovenian	Mešanica dveh linij humanih celic raka trebušne slinavke stabilno transducirane z retrovirusnim vektorjem za mišji gen *alfa-(1,3)-galaktozil transferaze*	Zdravljenje raka trebušne slinavke
Spanish	Mezcla de dos líneas celulares alogénicas humanas de cáncer de páncreas transducidas de forma estable con un vector retroviral que codifica el gen α-*(1,3)-galactosiltransferasa* murino	Tratamiento del cáncer de páncreas
Swedish	En blandning av två allogena mänskliga bukspottskörtelcancercellinjer som genomgått en stabil transduktion med en retroviral vector som kodar för den murina *alfa-(1,3)-galaktosyltransferasgenen*	Behandling av pankreascancer
Norwegian	Blanding av to allogene humane kreftcellelinjer fra bukspyttkjertel stabilt transdusert med retrovirus-vektor som koder for murint *alfa-(1,3)-galaktosyltransferase gen*	Behandling av pankreascancer
Icelandic	Blanda tveggja allogeneik manna briskrabbameins frumulína sem eru breyttar með stöðugum hætti með retróveiru ferju sem kóðar fyrir músa *alfa-(1,3)-galaktósýltransferasa* geninu	Meðferð briskrabbameins