



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of yttrium (⁹⁰Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1 for the treatment of pancreatic cancer

On 6 February 2009, orphan designation (EU/3/08/608) was granted by the European Commission to Immunomedics GmbH, Germany, for Yttrium (⁹⁰Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1 for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is a cancer of the pancreas, an organ that lies close to 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 severe and life-threatening disease.

What is the estimated number of patients affected by the pancreatic cancer?

At the time of designation pancreatic cancer affected approximately 1.3 in 10,000 people in the European Union (EU) *. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 65,000 people.

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 kill cancer cells). Satisfactory argumentation has been submitted by the sponsor to justify the assumption that Yttrium (⁹⁰Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1 might be of potential significant benefit for the treatment of pancreatic cancer because it has a new mechanism of action which may lead to a better overall outcome for the patients. 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?

Mucin 1 is a protein found on the membrane of many types of cells. However, abnormally high levels or changes in its structure have been associated with some types of cancer and mucin 1 is highly expressed in pancreatic cancer cells. This medicinal product contains an antibody that specifically binds to mucin 1 and is labelled with radioisotope Yttrium (⁹⁰Y)-DOTA.

Antibodies are proteins used by the immune system to identify and neutralize foreign proteins expressed by bacteria or viruses known as antigens. Antigens and antibodies have a lock-and-key relationship; an antibody can specifically recognize and bind only one antigen. As medicinal products,

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

antibodies can be used to identify specific antigens expressed only on cells of interest. This medicinal product is expected to recognise mucin 1 on pancreatic cancer cells, bind on them and deliver the radioisotope on the tumour. Radioisotopes are used to destroy cancer cells. The antibody is expected to help identify pancreatic cancer cells expressing mucin-1, and once the product is bound, Yttrium (^{90}Y)-DOTA is expected to destroy them.

What is the stage of development of this medicine?

The effects of Yttrium (^{90}Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1 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, Yttrium (^{90}Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1 was not authorised anywhere in the world for pancreatic cancer. Orphan designation of Yttrium (^{90}Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1 had been granted in the United States on 29 January 2004 for pancreatic cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 December 2008 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

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**Translations of the active ingredient and indication in all official EU languages,
Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Yttrium (⁹⁰ Y)-DOTA-radiolabelled humanized monoclonal antibody against mucin 1	Treatment of pancreatic cancer
Bulgarian	Итрий-90 ибритумомаб-DOTA хуманизирано моноклонално антитяло against mucin 1	Лечение на рак на панкреаса
Czech	Radioaktivním yttrium 90-DOTA značená lidská monoklonální protilátka proti mucinu 1	Léčba karcinomu pankreatu
Danish	Yttrium-90 radioaktivt-DOTA humant monoklonalt antistof mod mucin 1	Behandling af pancreascancer
Dutch	Yttrium-90 gelabeld-DOTA humaan monoklonaal antilichaam tegen mucin 1	Behandeling van pancreaskanker
Estonian	Üttrium-90-ga radiomärgistatud-DOTA mucin 1 vastane inimese monoklonaalne antikeha	Pankreasevähi ravi
Finnish	Yttrium (⁹⁰ Y): llä radioaktiivisesti merkitty DOTA-humanisoitu monoklonaalinen vasta-aine musiini 1:ä vastaan	Haimasyövän hoito
French	Anticorps monoclonal humain contre la mucine 1, marqué à l'Yttrium 90-DOTA	Traitement du cancer pancréatique
German	Yttrium-90-markiertem-DOTA humaner monoklonaler Antikörper gegen mucin 1	Behandlung des Pankreaskarzinoms
Greek	Ραδιοσημασμένο με ύτριο (90Y)-DOTA ανθρώπινο μονοκλωνικό αντίσωμα κατά mucin 1	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Yttrium-90 izotóppal jelzett-DOTA mucin 1 elleni humán monoklonális ellenanyag	Hasnyálmirigyrák kezelése
Italian	anticorpo monoclonale umanizzato contro la mucina 1, radiomarcato con ittrio-90-DOTA	Trattamento del cancro pancreatico
Latvian	Radioaktīvā izotopa itrija-90 - DOTA cilvēka monoklonālās anti vielas pret mucīnu 1	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Radioaktyviu itriu (90Y)-DOTA žymėtas žmogaus monokloninis antikūnas prieš mucina-1	Kasos vėžio gydymas
Maltese	Antikorp monoklonali umanizzat b'marka radjuattiva ta' yttrium-90-DOTA, kontra mucin 1	Kura tal-kanċer tal-frixa
Polish	Znakowane radioizotopem itru-90-DOTA ludzkie monoklonalne przeciwciało skierowane przeciwko mucin 1	Leczenie raka trzustki
Portuguese	Anticorpo monoclonal humano anti mucina 1, marcado com ítrio-90-DOTA radioactivo	Tratamento do cancro do pâncreas
Romanian	Anticorp monoclonal umanizat anti mucin 1 marcat radioactiv cu Ytriu-90-DOTA	Tratamentul cancerului pancreatic

Slovak	Rádioaktívnym ytriom-(90Y)-DOTA označená ľudská monoklonná protilátka proti mucínu 1	Liečba rakoviny pankreasu
Slovenian	Človeška monoklonska protitelesa proti mucin 1, označenim z Itrijem-90 -DOTA	Zdravljenje raka trebušne slinavke
Spanish	Anticuerpo monoclonal humanizado contra mucina 1, radiomarcado con itrio (90Y)-DOTA	Tratamiento del cáncer de páncreas
Swedish	Radioaktiv märkning av med yttrium 90 (90Y)-DOTA Human monoklonal antikropp mot mucin 1	Behandling av pankreascancer
Norwegian	Yttrium (90Y)-DOTA-radiolabeled Humant monoklonalt antistoff mot mucin 1	Behandling av pankreascancer
Icelandic	Yttrium (90Y)-DOTA-geislamerktu manna einstofna mótefni gegn mucíni 1	Meðferð briskrabbameins