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Public summary of opinion on orphan designation

Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2 for the treatment of pancreatic cancer

On 27 February 2017, orphan designation (EU/3/17/1838) was granted by the European Commission to Opsona Therapeutics Ltd, Ireland, for humanised IgG4 monoclonal antibody to the human toll-like receptor type 2 (also known as OPN-305) 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. 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. Laboratory data indicate that, when this medicine is used in combination with the cancer medicines gemcitabine and paclitaxel (attached to a human protein called

*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 515,700,000 (Eurostat 2017).

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albumin), it could reduce tumour size. These assumptions 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 a monoclonal antibody (a type of protein), that has been designed to attach to another protein called the human toll-like receptor type 2 (TLR2). TLR2 is part of the immune system (the body's natural defences) and may play a role in the spread of cancer. By attaching to TLR2, this medicine is expected to block its activity, thereby reducing cancer spread.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with pancreatic cancer had been started.

At the time of submission, the medicine 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 19 January 2017 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:

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	Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2	Treatment of pancreatic cancer
Bulgarian	Хуманизирано IgG4 моноклонално антитяло срещу човешкия Toll-like рецептор 2	Лечение на рак на панкреаса
Croatian	Humanizirano IgG4 monoklonsko protutijelo za ljudski toll-like receptor tipa 2	Liječenje raka gušterače
Czech	Humanizovaná IgG4 monoklonální protilátka proti humánnímu Toll-like receptoru typu 2	Léčba karcinomu pankreatu
Danish	Humaniseret monoklonalt antistof af typen IgG4 rettet mod den humane Toll-like receptor 2	Behandling af pancreascancer
Dutch	Gehumaniseerde monoklonale IgG4-antilichamen tegen humane Toll-like receptoren type 2	Behandeling van pancreaskanker
Estonian	Inimese 2. tüüpi toll-like retseptori humaniseeritud IgG4 monoklonaalne antikeha	Pankreasevähi ravi
Finnish	Humaanin ”Toll-like” reseptori tyyppi 2:n humanisoitu monoklonaalinen IgG4-luokan vasta-aine	Haimasyövän hoito
French	Anticorps monoclonal IgG4 humanisé dirigé contre le récepteur toll-like de type 2 (TLR2) humain	Traitement du cancer pancréatique
German	Humanisierter monoklonaler IgG4-Antikörper gegen den humanen Toll-like-Rezeptor Typ 2	Behandlung des Pankreaskarzinoms
Greek	Εξανθρωπισμένο μονοκλωνικό αντίσωμα IgG4 στον ανθρώπινο υποδοχέα TLR2 (Toll-like receptor)	Θεραπεία καρκίνου του παγκρέατος
Hungarian	2-es típusú, humán Toll-like receptor elleni humanizált IgG4 monoklonális antitest	Hasnyálmirigyrák kezelése
Italian	Anticorpo monoclonale IgG4 umanizzato verso il recettore Toll-like umano di tipo 2	Trattamento del cancro pancreatico
Latvian	Humanizētas IgG4 monoklonālās antivielas pret cilvēka Toll-līdzīgo 2.tipa receptoru	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Humanizuotas IgG4 monokloninis antikūnas, veikiantis žmogaus „toll-like“ 2 tipo receptorius	Kasos vėžio gydymas
Maltese	Antikorp monoklonali IgG4 umanizzat għar-riċettur tat-tip 2 “toll-like” uman	Kura tal-kanċer tal-frixa
Polish	Humanizowane przeciwciało monoklonalne IgG4 specyficzne względem ludzkiego receptora Toll-like typu 2-go	Leczenie raka trzustki
Portuguese	Anticorpos monoclonais IgG4 humanizados para o receptor “Toll-like” humano tipo 2	Tratamento do carcinoma do pâncreas

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Anticorp monoclonal IgG4 umanizat îndreptat împotriva receptorului uman „Toll-like” de tip 2	Tratamentul cancerului pancreatic
Slovak	Humanizovaná IgG4 monoklonová protilátka proti ľudskému toll-like receptoru typu 2	Liečba rakoviny pankreasu
Slovenian	Humanizirano monoklonsko protitelo IgG4 proti človeškemu Toll-like receptorju tipa 2	Zdravljenje raka trebušne slinavke
Spanish	Anticuerpo monoclonal humanizado IgG4 frente al receptor humano toll-like de tipo 2	Tratamiento del cáncer de páncreas
Swedish	Humaniserad IgG4 monoklonal antikropp mot human ”Toll-like” receptor 2	Behandling av pankreascancer
Norwegian	Humanisert IgG4 monoklonalt antistoff mot human “Toll-like” reseptor type 2	Behandling av pankreascancer
Icelandic	Mannaaðlagað IgG4 einstofna mótefni gegn manna „Toll-líkum” viðtaka af tegund 2	Meðferð briskrabbameins