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EMA/COMP/438204/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Chimeric monoclonal antibody against claudin-18 splice variant 2 for the treatment of pancreatic cancer

On 5 August 2013, orphan designation (EU/3/13/1177) was granted by the European Commission to Ganymed Pharmaceuticals AG, Germany, for chimeric monoclonal antibody against claudin-18 splice variant 2 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 approximately 1.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 56,000 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 509,000,000 (Eurostat 2013).

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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 studies in experimental models suggest that it may improve the survival of patients, when it is used in combination with a chemotherapy medicine called gemcitabine. 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?

Claudin-18 splice variant 2 is a protein found in the cells of the ducts of the pancreas, where it helps the cells to stick to each other. In patients with pancreatic cancer, this protein is produced in large amounts and is thought to be involved in the survival and spread of the cancer cells.

The medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a part of the claudin-18 splice variant 2 protein in the cancer cells. By attaching to this protein, this medicine is expected to stimulate the immune system (the body's natural defences) to kill the cancer cells, slowing down the spread of the cancer.

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. Orphan designation had been granted in the EU for the treatment of gastric cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2013 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	Chimeric monoclonal antibody against claudin-18 splice variant 2	Treatment of pancreatic cancer
Bulgarian	Химерно моноклонално антитяло към клаудин-18 сплайс-вариант 2	Лечение на рак на панкреаса
Croatian	Kimerično monoklonsko protutijelo protiv klaudina 18 tipa 2	Liječenje raka gušterače
Czech	Chimerická monoklonální protilátka proti claudinu-18, splice varianta 2	Léčba karcinomu pankreatu
Danish	Chimerisk monoklonalt antistof mod claudin-18 splice variant 2	Behandling af pancreascancer
Dutch	Chimeer monoklonaal antilichaam gericht tegen claudin-18 splice-variant 2	Behandeling van pancreaskanker
Estonian	Claudin-18 Splice 2 variandi vastane kimäärne monoklonaalne antikeha	Pankreasevähi ravi
Finnish	Klaudiini-18:n silmukointivariantti 2:n kimeerinen monoklonaalinen vasta-aine	Haimasyövän hoito
French	Anticorps monoclonal chimérique contre le variant 2 d'épissage de claudine 18	Traitement du cancer pancréatique
German	Chimärer monoklonaler Antikörper gegen Claudin-18 Splice Variante 2	Behandlung des Pankreaskarzinoms
Greek	Χιμαιρικό μονοκλωνικό αντίσωμα έναντι του παραγώγου διαφορικού ματίσματος 2 της κλωντίνης-18	Θεραπεία καρκίνου του παγκρέατος
Hungarian	Claudin-18-splice variáns 2 elleni kimera monoklonális antitest	Hasnyálmirigyrák kezelése
Italian	Anticorpo monoclonale chimerico contro la variante di giunzione 2 della claudina-18	Trattamento del cancro pancreatico
Latvian	Himēriska monoklonāla antiviela pret klaudīna-18 savīto 2. variantu	Aizkuņģa dziedzera vēža ārstēšana
Lithuanian	Chimerinis monokloninis antikūnas prieš klaudino-18 splaisingo 2 variantą	Kasos vėžio gydymas
Maltese	Antikorp monoklonali kimeriku kontra claudin-18 splice variant 2	Kura tal-kanċer tal-frixa
Polish	Chimeryczne przeciwciało monoklonalne przeciw klaudynie-18 o wariancie splicingowym 2	Leczenie raka trzustki
Portuguese	Anticorpo monoclonal quimérico anti variante 2 do Splice de claudin-18	Tratamento do cancro do pâncreas
Romanian	Anticorp monoclonal chimeric anticlaudina18 varianta de joncţiune 2	Tratamentul cancerului pancreatic

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Chimérická monoklonálna protilátka proti klaudínu-18, splice variant 2	Liečba rakoviny pankreasu
Slovenian	Kimerno monoklonsko protitelo proti klavdinu-18, različica spojitve 2	Zdravljenje raka trebušne slinavke
Spanish	Anticuerpo monoclonal quimérico contra la variante 2 del empalme del gen claudin-18	Tratamiento del cáncer de páncreas
Swedish	Chimär monoklonal antikropp mot claudin-18, splicevariant 2	Behandling av pankreascancer
Norwegian	Kimært monoklonalt antistoff mot claudin-18 spleisevariant 2	Behandling av pankreascancer
Icelandic	Einstofna blendingsmótefni gegn splæsiafbrigði 2 af claudín-18-próteini	Meðferð briskrabbameins