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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 gastric cancer

On 26 November 2010, orphan designation (EU/3/10/803) 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 gastric cancer.

What is gastric cancer?

Gastric cancer is a cancer that starts in the stomach, generally in the glandular cells lining the inside of the stomach. Gastric cancer is often detected late as the early signs of the disease are the same as those of less serious stomach conditions (heartburns, gas, excessive belching). At a later stage, gastric cancer causes unexplained weight loss, loss of appetite and general decline in health. Bleeding can occur, leading to anaemia (low red blood cell counts). Men are about twice as likely to develop the disease as women.

Gastric cancer is a serious and life-threatening illness that is associated with shortened life expectancy.

What is the estimated number of patients affected by the condition?

At the time of designation, gastric cancer affected approximately 3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 152,000 people, and is below the threshold 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, patients with gastric cancer were treated first with surgery to remove part of or the whole stomach. Chemotherapy (medicines to treat cancer) was generally used after surgery. Many chemotherapy medicines were authorised in the EU for use in gastric cancer, such as docetaxel, doxorubicin, capecitabine, carmustine, epirubicin, 5-fluorouracil, mitomycin C and trastuzumab. They were often used in combination.

[*]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 506,500,000 (Eurostat 2010).

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The sponsor has provided sufficient information to show that chimeric monoclonal antibody against claudin-18 splice variant 2 might be of significant benefit for patients with gastric cancer because it works in a different way to existing treatments and early studies in experimental models show that it might improve the treatment of patients with this condition. 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 glands lining the inside of the stomach, where it helps the gastric cells to stick to each other. In patients with gastric cancer, this protein is much more widespread 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 gastric cancer cells. By attaching to this protein, this medicine is expected to block the growth of cancer cells, slowing down the spread of the cancer.

What is the stage of development of this medicine?

The effects of chimeric monoclonal antibody against claudin-18 splice variant 2 have been evaluated in experimental models.

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

At the time of submission, the medicinal product was not authorised anywhere in the EU for gastric 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 9 September 2010 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 European Union) 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 gastric cancer
Bulgarian	Химерно моноклонално антитяло към Клаудин-18 сплайс-вариант 2	Лечение на карцином на стомаха
Czech	Chimerická monoklonální protilátka proti claudinu-18, splice varianta 2	Léčba karcinomu žaludku
Danish	Chimerisk monoklonalt antistof mod claudin-18 splice variant 2	Behandlingen af cancer ventriculi
Dutch	Chimeer monoklonaal antilichaam gericht tegen claudin-18 splice-variant 2	Behandeling van maagkanker
Estonian	Claudin-18 Splice 2. variandi vastane kimäärne monoklonaalne antikeha	Maovähi ravi
Finnish	Klaudiini-18:n silmukointivariantti 2:n kimeerinen monoklonaalinen vasta-aine	Mahasyövän hoito
French	Anticorps monoclonal chimérique contre le variant 2 d'épissage de claudine 18	Traitement du cancer gastrique
German	Chimärer monoklonaler Antikörper gegen Claudin-18 Splice Variante 2	Behandlung von Magenkrebs
Greek	Χιμαιρικό μονοκλωνικό αντίσωμα έναντι claudin-18 παραλλαγή ένωσης 2	Θεραπεία του γαστρικού καρκίνου
Hungarian	Kiméra monoklonális ellenanyag claudin-18 splice variant 2 ellen	Gyomorrák kezelése
Italian	Anticorpo monoclonale chimerico contro la variante di giunzione 2 della claudina-18	Trattamento del cancro gastrico
Latvian	Himēriska monoklonāla antiviela pret klaudīna-18 savīto 2. variantu	Kuņģa vēža ārstēšana
Lithuanian	Chimerinis monokloninis antikūnas prieš klaudino-18 splaisingo 2 variantą	Skrandžio vėžio gydymas
Maltese	Antikorp monoklonali kimeriku kontra claudin-18 splice variant 2	Kura tal-kanċer gastriku
Polish	Chimeryczne przeciwciało monoklonalne przeciw klaudynie-18 o wariancie splicingowym 2	Leczenie raka żołądka
Portuguese	Anticorpo monoclonal quimérico anti variante 2 do Splice de claudin-18	Tratamento do carcinoma gástrico
Romanian	Anticorp monoclonal chimeric anti-claudin-18 variantă splice 2	Tratamentul cancerului gastric
Slovak	Chimérická monoklonálna protilátka proti klaudínu-18, splice variant 2	Liečba rakoviny žalúdka
Slovenian	Kimerno monoklonsko protitelo proti klavdinu-18, različica spojitve 2	Zdravljenje karcinoma želodca

[1] At the time of designation

Spanish	Anticuerpo monoclonal quimérico contra la variante 2 del empalme del gen claudin-18	Tratamiento del cáncer de estómago
Swedish	Chimär monoklonal antikropp mot claudin-18, splicevariant 2	Behandling av magcancer
Norwegian	Kimært monoklonalt antistoff mot claudin-18 spleisevariant 2.	Behandling av magekreft
Icelandic	Einstofna blendingsmótefni gegn splæsiafbrigði 2 af claudin-18-próteini	Meðferð við magakrabbameini