9 February 2015
EMA/COMP/477962/2010 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Maytansinoid-conjugated humanised monoclonal antibody against CD56 for the treatment of small cell lung cancer

First publication	12 October 2010
Rev.1: sponsor's change of address	13 March 2013
Rev.2: withdrawal from the Community Register	9 February 2015

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2014 on request of the Sponsor.

On 1 October 2010, orphan designation (EU/3/10/792) was granted by the European Commission to ImmunoGen Europe Limited, United Kingdom, for maytansinoid-conjugated humanised monoclonal antibody against CD56 for the treatment of small cell lung cancer.

What is small cell lung cancer?

Small cell lung cancer is a type of lung cancer that usually develops in the central part of the lungs, and in which the cancer cells are small compared with other types of lung cancer. Small cell lung cancer is almost always caused by smoking. The cancer is difficult to detect in the early stages of the disease, and the majority of the patients are diagnosed when the cancer has spread and cannot be removed by surgery.

Small cell lung cancer is a life-threatening disease that is associated with poor long-term survival.

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

At the time of designation, small cell lung cancer affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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, several medicines were authorised in the EU for the treatment of small cell lung cancer. The choice of treatment depended mainly on how advanced the disease was. Treatments included chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that maytansinoid-conjugated humanised monoclonal antibody against CD56 might be of significant benefit for patients with small cell lung cancer because it works in a different way to existing treatments and because early studies show that it might be used in combination with existing treatments to improve the outcome 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?

Maytansinoid-conjugated humanised monoclonal antibody against CD56 is made up of two parts:

- a monoclonal antibody against CD56, a type of protein that has been designed to recognise and attach to a specific structure (antigen) called CD56. CD56 is a receptor that is found at high levels on the surface of small cell lung cancer cells;
- a maytansinoid called DM1. This is a 'cytotoxic' substance that kills cancer cells when they attempt to divide.

The antibody part allows the medicine to attach to CD56 on the surface of lung cancer cells. The antibody-maytansinoid complex then enters the cancerous cells, where the maytansinoid is released to exert its anti-tumour effect by blocking cell division, leading to cell death. This is expected to slow down the growth or cause the shrinkage of small cell lung cancer tumours.

What is the stage of development of this medicine?

The effects of maytansinoid-conjugated humanised monoclonal antibody against CD56 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 small cell lung cancer were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for small cell lung 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 8 July 2010 recommending the granting of this designation.

*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.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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	Maytansinoid-conjugated humanised monoclonal antibody against CD56	Treatment of small cell lung cancer
Bulgarian	Мейтензиноид-конюгирано хуманизирано моноклонално антитяло срещу CD56	Лечение на дребноклетъчен карцином на белия дроб
Czech	Maytansinoid – konjugovaná humanizovaná monoklonální protilátka proti CD56	Léčba malobuněčného karcinomu plic
Danish	Maytansinoid-konjugeret humaniseret monoklonalt antistof mod CD56	Behandling af småcellet lungecancer
Dutch	Maytansinoïde-geconjugeerd gehumaniseerd monoclonaal antilichaam tegen CD56	Behandeling van kleincellig longcarcinoom
Estonian	Maitansinoidiga konjugeeritud humaniseeritud CD56-vastane monoklonaalne antikeha	Väikeserakulise kopsuvähi ravi
Finnish	Maytansinoidi-konjugoitu, humanisoitu monoklonaalinen CD56:n vasta-aine	Keuhkojen pienisolusyövän hoito
French	Anticorps monoclonal humanise immunoconjugué de maytansinoïde anti-CD56	Traitement du cancer du poumon à petites cellules
German	Maytansinoid-konjugierter, humanisierter, monoklonaler Antikörper gegen CD56	Behandlung des kleinzelligen Lungenkarzinoms
Greek	Συζευγμένο με Μαϊτανσινοειδές Ανθρωποποιημένο Μονοκλωνικό Αντίσωμα έναντι του CD56	Θεραπεία του μικροκυτταρικού καρκίνου του πνεύμονα
Hungarian	CD56-elleni, maytansinoid-konjugált humanizált monoklonális antitest	Kissejtes tüdőrák kezelése
Italian	Anticorpo monoclonale anti-CD56 umanizzato coniugato con maytansinoidi	Trattamento del cancro del polmone a piccole cellule (microcitoma)
Latvian	Ar meitenzīnoīdu konjugēta humanizēta monoklonāla antiviela pret CD56X	Sīkšūnu plaušu vēža ārstēšana
Lithuanian	Humanizuotas monokloninis antikūnas konjuguotas su meitansinoidu prieš CD56	Smulkialąstelinio plaučių vėžio gydymas
Maltese	Antikorp monoklonali umanizzat kontra CD56 konjugat ma' maytansinoid	Kura tal-kanċer tal-pulmun b'ċelloli żgħar
Polish	Koniugat majtanzynoidu z humanizowanym przeciwciałem monoklonalnym przeciw CD56	Leczenie raka drobnokomórkowego pluc
Portuguese	Anticorpo monoclonal humanizado conjugado com maitansinoide anti-CD56	Tratamento do carcinoma de pequenas células do pulmão
Romanian	Anticorp monoclonal umanizat conjugat cu maytansinoid împotriva CD56	Tratamentul cancerului pulmonar cu celule mici

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Humanizovaná monoklonálna protilátka proti CD56 konjugovaná s maytansinoidom	Liečba malobunkového karcinómu pľúc
Slovenian	Z majtanzinoidom konjugirano humanizirano monoklonsko protitelo proti CD56	Zdravljenje drobnocelicnega raka pljuc
Spanish	Anticuerpo monoclonal humanizado conjugado con maitansinoide frente a CD56	Carcinoma de pulmón de células pequeñas
Swedish	Maytansinoid-konjugerad humaniserad monoklonal antikropp mot CD56	Behandling av småcellig lungcancer
Norwegian	Maytansinoid-konjugert humanisert monoklonalt antistoff mot CD56	Behandling av småcellet lungekreft
Icelandic	Maytansínóíð samtengt, mannaaðlagað einstofna mótefni gegn CD56	Til meðferðar við lungnakrabbameini af smáfrumugerð