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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Maytansinoid-conjugated humanised monoclonal antibody against CD56 for the treatment of Merkel cell carcinoma

First publication	10 June 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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2014 on request of the Sponsor.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 9 June 2010, orphan designation (EU/3/10/746) was granted by the European Commission to ImmunoGen Europe Limited, United Kingdom, for maytansinoid-conjugated humanised monoclonal antibody against CD56 for the treatment of Merkel cell carcinoma.

What is Merkel cell carcinoma?

Merkel cell carcinoma (also known as cutaneous neuro-endocrine carcinoma) is a type of skin cancer that starts in 'neuro-endocrine' cells called Merkel cells. Neuro-endocrine cells release hormones into the blood when stimulated by the nervous system. Merkel cell carcinoma usually appears as a painless red-blue lump on the head and neck, but it can also be found on the arms and legs, and sometimes on the trunk.

Merkel cell carcinoma is different from other skin cancers in that it grows rapidly. The disease is life threatening because it starts to spread quickly to other parts of the body. Once this cancer has spread, it is associated with very poor long-term survival.

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

At the time of designation, Merkel cell carcinoma affected approximately 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 20,000 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, no satisfactory methods were authorised in the EU to treat Merkel cell carcinoma. Patients were treated with chemotherapy (medicines to treat cancer), surgery and radiotherapy (treatment with radiation). However, in most patients whose disease had spread, the disease tended to come back after treatment.

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 cancer cells of neuro-endocrine origin, including Merkel 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 cancerous Merkel cells. The antibody-maytansinoid complex then enters the cancerous cells, where the maytansinoid exerts its anti-tumour effect by blocking cell division. This is expected to slow down the growth of Merkel cell carcinoma.

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 Merkel cell carcinoma were ongoing.

At the time of submission, maytansinoid-conjugated humanised monoclonal antibody against CD56 was not authorised anywhere in the EU for Merkel cell carcinoma 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 3 March 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 Lichtenstein. 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Maytansinoid-conjugated humanised monoclonal antibody against CD56	Treatment of Merkel cell carcinoma
Bulgarian	Мейтензиноид-конюгирано хуманизирано моноклонално антитяло срещу CD56	Лечение на Меркелклетъчен карцином
Czech	Maytansinoid – konjugovaná humanizovaná monoklonální protilátka proti CD56	Léčba rakoviny z Merkelových buňek
Danish	Maytansinoid-konjugeret humaniseret monoklonalt antistof mod CD56	Behandling af Merkelcellekarcinom
Dutch	Maytansinoïde-geconjugueerd gehumaniseerd monoclonaal antilichaam tegen CD56	Behandeling van Merkelcelcarcinoom
Estonian	Maitansinoidiga konjugeeritud humaniseeritud CD56-vastane monoklonaalne antikeha	Merkelirakulise vähi ravi
Finnish	Maytansinoidi-konjugoitu, humanisoitu monoklonaalinen CD56:n vasta-aine	Merkelinsolukarsinooman hoito
French	Anticorps monoclonal humanise immunoconjugué de maytansinoïde anti- CD56	Traitement du carcinome à cellules de Merkel
German	Maytansinoid-konjugierter, humanisierter, monoklonaler Antikörper gegen CD56	Behandlung von Merkelzellkarzinomen
Greek	Συζευγμένο με Μαϊτανσινοειδές Ανθρώποποιημένο Μονοκλωνικό Αντίσωμα έναντι του CD56	Θεραπεία Καρκινώματος Κυττάρων Merkel
Hungarian	CD56-elleni, maytansinoid-konjugált humanizált monoklonális antitest	Merkel sejtes carcinoma kezelése
Italian	Anticorpo monoclonale anti-CD56 umanizzato coniugato con maytansinoidi	Trattamento del carcinoma delle cellule di Merkel
Latvian	Ar meitenzīnoīdu konjugēta humanizēta monoklonāla antivielā pret CD56X	Merkela šūnu karcinomas ārstēšana
Lithuanian	Humanizuotas monokloninis antikūnas konjuguotas su meitansinoidu prieš CD56	Merkelio ląstelių karcinomos gydymas
Maltese	Antikorp monoklonali umanizzat kontra CD56 konjugat ma' maytansinoid	Kura tal-karċinoma taċ-ċelloli ta' Merkel
Polish	Koniugat majtanzynoidu z humanizowanym przeciwciałem monoklonalnym przeciw CD56	Leczenie raka z komórek Merkla
Portuguese	Anticorpo monoclonal humanizado conjugado com maitansinoide anti-CD56	Tratamento de carcinoma de células de Merkel
Romanian	Anticorp monoclonal umanizat conjugat cu maytansinoid împotriva CD56	Tratamentul carcinomului cu celule Merkel
Slovak	Humanizovaná monoklonálna protilátka proti CD56 konjugovaná s maytansinoidom	Liečba rakoviny z Merkelových buniek
Slovenian	Z majtanzinoidom konjugirano humanizirano monoklonsko protitelo proti CD56	Zdravljenje karcinoma Merklvih celic

¹ At the time of designation

Language	Active ingredient	Indication
Spanish	Anticuerpo monoclonal humanizado conjugado con maitansinoide frente a CD56	Tratamiento del carcinoma de células de Merkel
Swedish	Maytansinoid-konjugerad humaniserad monoklonal antikropp mot CD56	Behandling av merkelcellskarcinom
Norwegian	Maytansinoid-konjugert humanisert monoklonalt antistoff mot CD56	Begandling av Merkelcellekarsinom
Icelandic	Maytansínóið samtengt, mannaaðlagað einstofna mótefni gegn CD56	Meðferð við Merkel frumu krabbameini