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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 multiple myeloma

First publication	2 March 2011
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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2014 on request of the Sponsor.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 23 February 2011, orphan designation (EU/3/10/835) was granted by the European Commission to ImmunoGen Europe Limited, United Kingdom, for maytansinoid-conjugated humanised monoclonal antibody against CD56 for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called plasma cells. Plasma cells in multiple myeloma are found in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes out of control, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of normal white blood cells, red blood cells and platelets (components that help the blood to clot), leading to complications such as anaemia (low red blood cell counts), bone pain and fractures, raised blood calcium levels and kidney disease.

Multiple myeloma is a debilitating and 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, multiple myeloma affected approximately 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 127,000 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 for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined with steroids to reduce the activity of the immune system, the body's natural defences. Radiotherapy (treatment with radiation) was considered to be very useful in treating pain and weakened bones. Interferon alfa, a protein normally produced by the body during viral infections, was sometimes used in combination with chemotherapy.

The sponsor has provided sufficient information to show that maytansinoid-conjugated humanised monoclonal antibody against CD56 might be of significant benefit for patients with multiple myeloma because it works in a different way to existing treatments, and early studies show that it might be used in combination with other treatments to improve the outcome of patients with this condition. 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?

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

- a monoclonal antibody, 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 myeloma cells;
- a maytansinoid called DM1, a 'cytotoxic' substance that kills cells when they attempt to divide.

The antibody part allows the medicine to attach to CD56 on the surface of cancer cells. The antibody-maytansinoid complex then enters the cancerous cells, where the maytansinoid is released to exert its cytotoxic effect by blocking cell division, leading to cell death. This is expected to slow down the growth or cause the shrinkage of multiple myeloma 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 the medicine in patients with multiple myeloma and other types of cancer were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for multiple myeloma. Orphan designation of maytansinoid-conjugated humanised monoclonal antibody against CD56 had been granted in the EU and the United States of America for Merkel cell carcinoma and small cell lung carcinoma.

*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 507,700,000 (Eurostat 2011).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 November 2010 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	Maytansinoid-conjugated humanised monoclonal antibody against CD56	Treatment of multiple myeloma
Bulgarian	Мейтензиноид-конюгирано хуманизирано моноклонално антитяло срещу CD56	Лечение на мултиплен миелом
Czech	Maytansinoid – konjugovaná humanizovaná monoklonální protilátka proti CD56	Léčba mnohočetného myelomu
Danish	Maytansinoid-konjugeret humaniseret monoklonalt antistof mod CD56	Behandling af multipelt myelom
Dutch	Maytansinoïde-geconjugeerd gehumaniseerd monoclonaal antilichaam tegen CD56	Behandeling van multipel myeloom
Estonian	Maitansinoidiga konjugeeritud humaniseeritud CD56-vastane monoklonaalne antikeha	Multiibelse müeloomi ravi
Finnish	Maytansinoidi-konjugoitu, humanisoitu monoklonaalinen CD56:n vasta-aine	Multippeli myelooman hoito
French	Anticorps monoclonal humanise immunoconjugué de maytansinoïde anti- CD56	Traitement du myélome multiple
German	Maytansinoid-konjugierter, humanisierter, monoklonaler Antikörper gegen CD56	Behandlung des multiplen Myeloms
Greek	Συζευγμένο με Μαϊτανσινοειδές Ανθρωποποιημένο Μονοκλωνικό Αντίσωμα έναντι του CD56	Θεραπευτική αγωγή πολλαπλού μυελώματος
Hungarian	CD56-elleni, maytansinoid-konjugált humanizált monoklonális antitest	Myeloma multiplex kezelése
Italian	Anticorpo monoclonale anti-CD56 umanizzato coniugato con maytansinoidi	Trattamento del mieloma multiplo
Latvian	Ar meitenzīnoīdu konjugēta humanizēta monoklonāla antiviela pret CD56X	Multiplās mielomas ārstēšana
Lithuanian	Humanizuotas monokloninis antikūnas konjuguotas su meitansinoidu prieš CD56	Dauginės mielomos gydymas
Maltese	Antikorp monoklonali umanizzat kontra CD56 konjugat ma' maytansinoid	Kura tal-mjeloma multipla
Polish	Koniugat majtanzynoidu z humanizowanym przeciwciałem monoklonalnym przeciw CD56	Leczenie szpiczaka mnogiego
Portuguese	Anticorpo monoclonal humanizado conjugado com maitansinoide anti-CD56	Tratamento do mieloma múltiplo
Romanian	Anticorp monoclonal umanizat conjugat cu maytansinoid împotriva CD56	Tratamentul mielomului multiplu
Slovak	Humanizovaná monoklonálna protilátka proti CD56 konjugovaná s maytansinoidom	Liečba mnohopočetného myelómu
Slovenian	Z majtanzinoidom konjugirano humanizirano monoklonsko protitelo proti CD56	Zdravljenje multiplega mieloma
Spanish	Anticuerpo monoclonal humanizado conjugado con maitansinoide frente a CD56	Tratamiento del mieloma múltiple

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Maytansinoid-konjugerad humaniserad monoklonal antikropp mot CD56	Behandling av multipelt myelom
Norwegian	Maytansinoid-konjugert humanisert monoklonalt antistoff mot CD56	Behandling av myelomatose
Icelandic	Maytansínóíð samtengt, mannaaðlagað einstofna mótefni gegn CD56	Meðferð við mergfrumuæxli