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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Chimeric monoclonal antibody against kappa myeloma antigen for the treatment of multiple myeloma

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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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2015 on request of the Sponsor.

On 22 May 2012, orphan designation (EU/3/12/962) was granted by the European Commission to Gregory Fryer Associates Ltd, United Kingdom, for chimeric monoclonal antibody against kappa myeloma antigen for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cells called plasma cells. Plasma cells 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 because it disrupts the normal functioning of the bone marrow, leads to bone destruction and causes kidney failure.



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

At the time of designation, multiple myeloma affected less than 2.9 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 148,000 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 submission of the application for orphan designation, several medicines were already 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 defenses. Where chemotherapy did not work, some patients received an allogeneic stem-cell transplant (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow). Radiotherapy (using radiation to kill cancer cells) was used to treat 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 chimeric monoclonal antibody against kappa myeloma antigen might be of significant benefit for patients with multiple myeloma because it works in a different way to existing treatments, and may be used in combination with other medicines 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?

Chimeric monoclonal antibody against kappa myeloma antigen is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific structure (an antigen) on the myeloma cell called 'kappa myeloma antigen'. Once attached, it is expected to activate cells of the immune system which will kill the cancerous plasma cells.

What is the stage of development of this medicine?

The effects of chimeric monoclonal antibody against kappa myeloma antigen 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 were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for multiple myeloma. Orphan designation of chimeric monoclonal antibody against kappa myeloma antigen had been granted in the United States of America for multiple myeloma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 January 2012 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 509,000,000 (Eurostat 2012).

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

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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 substance	Indication
English	Chimeric monoclonal antibody against kappa myeloma antigen	Treatment of multiple myeloma
Bulgarian	Химерно моноклонално антицяло срещу kappa миелом антиген	Лечение на множествен миелом
Czech	Chimerická monoklonální protilátka proti kappa myelom antigenu	Léčba mnohočetného myelomu
Danish	Kimært monoklonalt antistof mod kappa-myelom-antigen	Behandling af myelomatose
Dutch	Chimerisch monoklonaal antilichaam tegen kappa-myeloom-antigen	Behandeling van multipel myeloom
Estonian	Kimäärne monokloonne antikeha kappa müeloomi antigeeni vastu	Hulgimüeloomi ravi
Finnish	Kimeerinen monoklonaalinen kappaketjun myelooma-antigeenin (KMA) vasta-aine	Multippelin myelooman hoito
French	Anticorps monoclonal chimérique contre l'antigène du myélome kappa	Traitement du myélome multiple
German	Chimärer monoklonaler Antikörper gegen das Kappa-Myelom-Antigen	Behandlung des multiplen Myeloms
Greek	Χιμαϊρικό μονόκλωνο αντίσωμα έναντι του αντιγόνου κάππα του μυελώματος	Θεραπεία πολλαπλού μυελώματος
Hungarian	Kappa myeloma antigén elleni kiméra monoklonális antitest	A myeloma multiplex kezelése
Italian	Anticorpo monoclonale chimerico contro l'antigene kappa del mieloma	Trattamento del mieloma multiplo
Latvian	Himēriska monoklonāla antivielā, pret mielomas šūnu antigēnu kappa ķēdē	Multiplās mielomas ārstēšana
Lithuanian	Chimerinis monokloninis antikūnas, nukreiptas prieš kappa mielomos antigeną	Daugybinės mielomos gydymas.
Maltese	Antikorp monoklonali kimeriku kontra antigen kappa tal-majeloma	Kura ta' majeloma multipla
Polish	Chimeryczne przeciwciało monoklonalne przeciw antygenowi typu kappa szpiczaka	Leczenie szpiczaka mnogiego
Portuguese	Anticorpo monoclonal quimérico contra o antígeno capa do mieloma	Tratamento do mieloma múltiplo
Romanian	Anticorp monoclonal chimeric îndreptat împotriva antigenului mielomului kappa	Tratamentul mielomului multiplu
Slovak	Chimerická monoklonálna protilátka proti antigénu kappa myeolómu	Liečba mnohopočetného myeolómu
Slovenian	Himerno monoklonalno protitelo, ki deluje na kappa mielom antigen	Za zdravljenje multiplega mieloma
Spanish	Anticuerpo quimérico monoclonal contra el antígeno kappa del mieloma	Tratamiento de mieloma múltiple

¹ At the time of designation

Language	Active substance	Indication
Swedish	Chimär antikropp riktad mot antigenet kappa myelom	Behandling av multipelt myelom
Norwegian	Kimerisk monoklonalt antistoff mot kappa-myelomantigen	Behandling av multippelt myelom
Icelandic	Einklóna blendingsmótefni gegn mótefnavaka kappa mergæxla	Meðferð við mergæxlageri