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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Elotuzumab for the treatment of multiple myeloma

On 9 August 2012, orphan designation (EU/3/12/1037) was granted by the European Commission to Bristol-Myers Squibb Pharma EEIG, United Kingdom, for elotuzumab 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 approximately 1.6 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 81,000 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 defences. Where chemotherapy did not work, some patients

[*]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 506,300,000 (Eurostat 2011).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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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 elotuzumab might be of significant benefit for patients with multiple myeloma because it targets a specific protein found in high levels on the surface of the cancer cells, and preliminary studies indicated that when used in combination with other medicines it may 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?

Elotuzumab is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific structure in the body. Elotuzumab is expected to attach to a protein called human CD-2 subset 1 (CS1) that is found at high levels on the surface of myeloma cells.

When elotuzumab attaches to CS1 on the surface of myeloma cells, it is expected to activate certain cells of the immune system, called natural killer cells. These cells will then attach to the antibody and kill the cancer cells. 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 elotuzumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with multiple myeloma were ongoing.

At the time of submission, elotuzumab was not authorised anywhere in the world for multiple myeloma. Orphan designation of elotuzumab 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 July 2012 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:

Bristol-Myers Squibb Pharma EEIG
Uxbridge Business Park
Sanderson Road
Uxbridge UB8 1DH
United Kingdom
Telephone: +44 18 95 52 37 40
Telefax: +44 18 95 52 36 77
E-mail: medical.information@bms.com

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	Elotuzumab	Treatment of multiple myeloma
Bulgarian	Елотузумаб	Лечение на множествен миелом
Czech	Elotuzumab	Léčba mnohočetného myelomu
Danish	Elotuzumab	Behandling af myelomatose
Dutch	Elotuzumab	Behandeling van multipel myeloom
Estonian	Elotusumab	Multiipelmüeloomi ravi
Finnish	Elotutsumabi	Multippelin myelooman hoito
French	Elotuzumab	Traitement du myélome multiple
German	Elotuzumab	Behandlung des multiplen Myeloms
Greek	Ελοτουζουμάμπη	Θεραπεία πολλαπλού μυελώματος
Hungarian	Elotozumab	A myeloma multiplex kezelése
Italian	Elotuzumab	Trattamento del mieloma multiplo
Latvian	Elotuzumabs	Multiplās mielomas ārstēšana
Lithuanian	Elotuzumabas	Daugybinės mielomos gydymas.
Maltese	Elotuzumab	Kura ta' mjeloma multipla
Polish	Elotuzumab	Leczenie szpiczaka mnogiego
Portuguese	Elotuzumab	Tratamento do mieloma múltiplo
Romanian	Elotuzumab	Tratamentul mielomului multiplu
Slovak	Elotuzumab	Liečba mnohopočetného myelómu
Slovenian	Elotuzumab	Za zdravljenje multiplega mieloma
Spanish	Elotuzumab	Tratamiento de mieloma múltiple
Swedish	Elotuzumab	Behandling av multipelt myelom
Norwegian	Elotuzumab	Behandling av multippelt myelom
Icelandic	Elótúzúmab	Meðferð við mergæxlageri

[1] At the time of designation