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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Carfilzomib for the treatment of multiple myeloma

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Rev.1: transfer of sponsorship	12 August 2009
Rev.2: sponsor's change of address	12 November 2009
Rev.3: transfer of sponsorship	18 February 2013
Rev.4: transfer of sponsorship	27 June 2014
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.	

On 3 June 2008, orphan designation (EU/3/08/548) was granted by the European Commission to Interface International Consultancy Ltd, United Kingdom, for carfilzomib for the treatment of multiple myeloma.

The sponsorship was transferred as follows:

- to Nexus Oncology Ltd, United Kingdom, in October 2008,
- to Onyx Pharmaceuticals (UK) Ltd, United Kingdom, in November 2012 and
- to Amgen Europe BV, The Netherlands, in June 2014.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell 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 gets out of control, and results in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of the 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 life-threatening disease.

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

At the time of designation, multiple myeloma affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 65,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for orphan drug designation, several medicines were authorised for multiple myeloma in the European Union. The main treatment for multiple myeloma is chemotherapy (medicines to treat cancer) usually combined with steroids (a group of chemical substances, the so-called hormones, which have an effect on the activity of certain organs). Interferon alfa can also be used in combination with chemotherapy although the way it works in cancer treatment is not fully understood. Radiotherapy (using radiation to kill cancer cells) can be very useful to treat pain and weakened bones.

The sponsor has provided satisfactory documentation to justify the assumption that carfilzomib could be of benefit in the treatment of multiple myeloma because it may offer a new way of killing cancer cells and stopping tumour growth. This assumption will need to be confirmed at the time of a marketing authorisation, to maintain the orphan status of the medicine.

How is this medicine expected to work?

Carfilzomib is a proteasome inhibitor. It blocks the proteasome, a system in cells that breaks down proteins when they are no longer needed. When the proteins in the cancer cells, such as the proteins that control the growth of the cells, are not broken down, the cells are affected and they eventually die. This is expected to reduce the number of multiple myeloma cells.

What is the stage of development of this medicine?

The effects of cafilzomib 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, carfilzomib was not authorised anywhere in the world for the treatment of multiple myeloma, or designated as 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 April 2008 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 502,800,000 (Eurostat 2008).

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	Carfilzomib	Treatment of multiple myeloma
Bulgarian	Карфилзомиб	Лечение на мултиплен миелом
Croatian	Karfilzomib	Liječenje multiplog mijeloma
Czech	Karfilzomib	Léčba mnohočetného myelomu
Danish	Carfilzomib	Behandling af multipelt myelom
Dutch	Carfilzomib	Behandeling van multipel myeloom
Estonian	Karfilzomiib	Multiibelse imüeloomi ravi
Finnish	Karfiltsomibi	Multippeli myelooman hoito
French	Carfilzomib	Traitement du myélome multiple
German	Carfilzomib	Behandlung des multiplen Myeloms
Greek	Καρφιλζομίμπη	Θεραπευτική αγωγή πολλαπλού μυελώματος
Hungarian	Carfilzomib	Myeloma multiplex kezelése
Italian	Carfilzomib	Trattamento del mieloma multiplo
Latvian	Karfilzomibs	Multiplās mielomas ārstēšana
Lithuanian	Karfilzomibas	Dauginės mielomos gydymas
Maltese	Carfilzomib	Kura tal-mjeloma multipla
Polish	Carfilzomib	Leczenie szpiczaka mnogiego
Portuguese	Carfilzomib	Tratamento do mieloma múltiplo
Romanian	Carfilzomib	Tratamentul mielomului multiplu
Slovak	Karfilzomib	Liečba mnohopočetného myelómu
Slovenian	Karfilzomib	Zdravljenje multiplega mieloma
Spanish	Carfilzomib	Tratamiento del mieloma múltiple
Swedish	Karfilzomib	Behandling av multipelt myelom
Norwegian	Karfilzomib	Behandling av myelomatose
Icelandic	Carfilzómíb	Meðferð við mergfrumuæxli

[1] At the time of designation