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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pomalidomide for the treatment of multiple myeloma

First publication	13 October 2009
Rev.1: sponsor's change of address	20 June 2011
Rev.2: information about Marketing Authorisation	11 September 2013
Rev.3: administrative update	14 October 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 8 October 2009, orphan designation (EU/3/09/672) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for pomalidomide 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 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 life-threatening disease that leads to poor long-term survival.

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

At the time of designation, multiple myeloma affected approximately 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 111,000 people*, and is below the

*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 504,800,000 (Eurostat 2009).

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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 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 pomalidomide might be of significant benefit for patients with multiple myeloma because it might be used in patients who do not respond to existing treatments. In addition, the medicine will be available as capsules, whereas some existing treatments need to be given by injection. 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?

Pomalidomide is an immunomodulating agent. This means that it affects the activity of the immune system. Pomalidomide is expected to work in a number of ways in multiple myeloma, in a similar way to other immunomodulating agents such as lenalidomide and thalidomide: it is expected to block the development of tumour cells, by preventing the growth of blood vessels within tumours and thereby reducing the supply of oxygen and nutrients to the cancer cells; it is also expected to stimulate some of the specialised cells of the immune system to attack the cancerous cells.

What is the stage of development of this medicine?

The effects of pomalidomide 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, pomalidomide was not authorised anywhere in the EU for multiple myeloma. Orphan designation of pomalidomide had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2009 recommending the granting of this designation.

Update: Pomalidomide (Imnovid, previously Pomalidomide Celgene) has been authorised in the EU since 5 August 2013. Imnovid in combination with dexamethasone is indicated in the treatment of adult patients with relapsed and refractory multiple myeloma who have received at least two prior treatment regimens, including both lenalidomide and bortezomib, and have demonstrated disease progression on the last therapy.

More information on Imnovid can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

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	Pomalidomide	Treatment of multiple myeloma
Bulgarian	Помалидомид	Лечение на мултиплен миелом
Czech	Pomalidomid	Léčba mnohočetného myelomu
Danish	Pomalidomid	Behandling af multipelt myelom
Dutch	Pomalidomide	Behandeling van multipel myeloom
Estonian	Pomalidomiid	Multiibelse müeloomi ravi
Finnish	Pomalidomidi	Multippeli myelooman hoito
French	Pomalidomide	Traitement du myélome multiple
German	Pomalidomid	Behandlung des multiplen Myeloms
Greek	Πομαλιδομίδη	Θεραπευτική αγωγή πολλαπλού μυελώματος
Hungarian	Pomalidomid	Myeloma multiplex kezelése
Italian	Pomalidomide	Trattamento del mieloma multiplo
Latvian	Pomalidomīds	Multiplās mielomas ārstēšana
Lithuanian	Pomalidomidas	Dauginės mielomos gydymas
Maltese	Pomalidomide	Kura tal-mjeloma multipla
Polish	Pomalidomid	Leczenie szpiczaka mnogiego
Portuguese	Pomalidomida	Tratamento do mieloma múltiplo
Romanian	Pomalidomidă	Tratamentul mielomului multiplu
Slovak	Pomalidomid	Liečba mnohopočetného myelómu
Slovenian	Pomalidomid	Zdravljenje multiplega mieloma
Spanish	Pomalidomida	Tratamiento del mieloma múltiple
Swedish	Pomalidomid	Behandling av multipelt myelom
Norwegian	Pomalidomid	Behandling av myelomatose
Icelandic	Pómalídómíð	Meðferð við mergfrumuæxli

[1] At the time of designation