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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Panobinostat 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.	

On 8 November 2012, orphan designation (EU/3/12/1063) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for panobinostat 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 dysfunction.

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 not more than 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 163,000 people*, and is below

*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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
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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. 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 panobinostat might be of significant benefit for patients with multiple myeloma because early studies show that, when used in combination with existing chemotherapy treatment, it may be effective in treating the disease when it has come back or does not respond to other treatments alone. 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?

Panobinostat is expected to work by blocking the activity of proteins called histone deacetylases (HDACs), which are involved in turning genes 'on' and 'off' within cells. In multiple myeloma, panobinostat is expected to have several actions, including keeping the genes that suppress the division and growth of the cancer cells switched 'on'. This is expected to lead to a reduction in the growth and division of the cancer cells, thereby slowing down the growth of the cancer.

What is the stage of development of this medicine?

The effects of panobinostat have been evaluated in experimental models.

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

At the time of submission, panobinostat was not authorised anywhere in the EU for multiple myeloma. Orphan designation of panobinostat 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 5 October 2012 recommending the granting of this designation.

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:

Novartis Europharm Limited
Frimley Business Park
Camberley GU16 7SR
United Kingdom
Tel. +41 61 324 11 11 (Switzerland)
E-mail: orphan.enquiries@novartis.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 substance	Indication
English	Panobinostat	Treatment of multiple myeloma
Bulgarian	Панобиностат	Лечение на мултиплен миелом
Czech	Panobinostat	Léčba mnohočetného myelomu
Danish	Panobinostat	Behandling af multipelt myelom
Dutch	Panobinostat	Behandeling van multipel myeloom
Estonian	Panobinostaat	Multiibelse müeloomi ravi
Finnish	Panobinostaatti	Multippeli myelooman hoito
French	Panobinostat	Traitement du myélome multiple
German	Panobinostat	Behandlung des multiplen Myeloms
Greek	Πανομπινοστάτη	Θεραπεία πολλαπλού μυελώματος
Hungarian	Panobinosztát	Myeloma multiplex kezelése
Italian	Panobinostat	Trattamento del mieloma multiplo
Latvian	Panobinostats	Multiplās mielomas ārstēšana
Lithuanian	Panobinostatas	Dauginės mielomos gydymas
Maltese	Panobinostat	Kura tal-mjeloma multipla
Polish	Panobinostat	Leczenie szpiczaka mnogiego
Portuguese	Panobinostat	Tratamento do mieloma múltiplo
Romanian	Panobinostat	Tratamentul mielomului multiplu
Slovak	Panobinostat	Liečba mnohopočetného myelómu
Slovenian	Panobinostat	Zdravljenje multiplega mieloma
Spanish	Panobinostato	Tratamiento del mieloma múltiple
Swedish	Panobinostat	Behandling av multipelt myelom
Norwegian	Panobinostat	Behandling av myelomatose
Icelandic	Panóbínóstat	Meðferð við mergfrumuæxli

[1] At the time of designation