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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Panobinostat for the treatment of Hodgkin's lymphoma

First publication	3 March 2010
Rev.1: withdrawal from the Community Register	3 May 2012
Rev.2: sponsor's change of address	5 February 2015
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Please note that this product was withdrawn from the Community register of designated orphan medicinal products in April 2012 on request of the sponsor.

On 2 February 2010, orphan designation (EU/3/09/721) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for panobinostat for the treatment of Hodgkin's lymphoma.

What is Hodgkin's lymphoma?

Hodgkin's lymphoma is a type of cancer of the lymphatic system, a network of vessels that transport lymph from tissues through the lymph nodes and into the bloodstream. Because lymph nodes are found throughout the body, the cancer can begin in almost any part of the body. In Hodgkin's lymphoma, white blood cells in the lymphatic system multiply too quickly and live for too long. These cancer cells can spread through the lymphatic system to other lymph nodes or through the bloodstream to other organs such as the spleen, where they can form new tumours.

Many people with Hodgkin's lymphoma can be cured if the disease is found and treated early. However, despite the available treatments, Hodgkin's lymphoma remains a serious and life-threatening disease, mainly because it leads to poor survival in patients whose disease does not respond to treatment or has come back after previous treatment.

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

At the time of designation, Hodgkin's lymphoma affected between 1 and 4 in 10,000 people in the European Union (EU). This was equivalent to a total of between 51,000 and 203,000 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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, several medicines were authorised for the treatment of Hodgkin's lymphoma in the EU. The main treatments for Hodgkin's lymphoma included chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation). Autologous bone marrow transplantation was also used when the disease had not responded to treatment or had come back after treatment. This is a complex procedure where the bone marrow of the patient is destroyed and replaced with healthy bone marrow previously obtained from the same patient.

The sponsor has provided sufficient information to show that panobinostat might be of significant benefit for patients with Hodgkin's lymphoma because early studies indicate that it might improve the treatment of patients with this condition, particularly patients whose disease has come back after previous treatment. 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, which are involved in turning genes 'on' and 'off' within cells. In Hodgkin's lymphoma, panobinostat is expected to keep the genes that suppress the division and growth of the tumour cells switched 'on'. This is expected to lead to a reduction in the growth and division of the lymphoma cells.

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 the designated product in patients with Hodgkin's lymphoma were ongoing.

At the time of submission, panobinostat was not authorised anywhere in the EU for Hodgkin's lymphoma. Orphan designation of panobinostat had been granted in the United States of America for Hodgkin's lymphoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 December 2009 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 506,300,000 (Eurostat 2010).

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 Community) 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, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Panobinostat	Treatment of Hodgkin's lymphoma
Bulgarian	Панобиностат	Лечение на лимфом на Хочкин
Czech	Panobinostat	Léčba Hodgkinova lymfomu
Danish	Panobinostat	Behandling af Hodgkin lymfom
Dutch	Panobinostat	Behandeling van Hodgkin lymfoom
Estonian	Panobinostaat	Hodgkini lümfoomi ravi
Finnish	Panobinostaatti	Hodgkinin lymfooman hoito
French	Panobinostat	Traitement du lymphome de Hodgkin
German	Panobinostat	Behandlung des Hodgkin-Lymphoms
Greek	Πανομπινοστάτη	Θεραπεία του λεμφώματος Hodgkin
Hungarian	Panobinosztát	Hodgkin lymphoma kezelése
Italian	Panobinostat	Trattamento del linfoma di Hodgkin
Latvian	Panobinostats	Hodžkina limfomas ārstēšana
Lithuanian	Panobinostatas	Hodžkino limfomos gydymas
Maltese	Panobinostat	Kura tal-limfoma ta' Hodgkin
Polish	Panobinostat	Leczenie chłoniaka Hodgkina (ziarnicy złośliwej)
Portuguese	Panobinostato	Tratamento do linfoma de Hodgkin
Romanian	Panobinostat	Tratamentul limfomului Hodgkin
Slovak	Panobinostat	Liečba lymfómu Hodgkinovho typu
Slovenian	Panobinostat	Zdravljenje Hodgkinovega limfoma
Spanish	Panobinostat	Tratamiento del linfoma de Hodgkin
Swedish	Panobinostat	Behandling av Hodgkin lymfom
Norwegian	Panobinostat	Behandling av Hodgkin-lymfom
Icelandic	Panóbínóstat	Meðferð við Hodgkins sjúkdómi