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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Entinostat for the treatment of Hodgkin's lymphoma

First publication	22 June 2010
Rev.1: sponsor's name change	5 June 2013
Rev.2: transfer of sponsorship	18 May 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.	

On 10 June 2010, orphan designation (EU/3/10/732) was granted by the European Commission to Nexus Oncology Ltd, United Kingdom, for entinostat for the treatment of Hodgkin's lymphoma.

In February 2013, Nexus Oncology Ltd changed name to Ockham Europe Limited.

The sponsorship was transferred to Syndax Limited, United Kingdom, in April 2015.

What is Hodgkin's lymphoma?

Hodgkin's lymphoma is a type of cancer of the lymphatic system, a network of vessels that transport fluid 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 treatments available, 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.

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 entinostat might be of significant benefit for patients with Hodgkin's lymphoma because it works in a different way to existing treatments and may represent an alternative treatment option for patients. 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?

Entinostat is expected to work by blocking the activity of proteins called class I histone deacetylases, which are involved in turning genes 'on' and 'off' within cells. In Hodgkin's lymphoma, entinostat 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 entinostat have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with entinostat in patients with Hodgkin's lymphoma were ongoing.

At the time of submission, entinostat was not authorised anywhere in the EU for Hodgkin's lymphoma or designated as an 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 3 February 2010 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 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 languages¹, Norwegian and Icelandic

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

¹ At the time of transfer of sponsorship