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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Belinostat for the treatment of malignant thymoma

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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 17 July 2013, orphan designation (EU/3/13/1151) was granted by the European Commission to Topotarget A/S, Denmark, for belinostat for the treatment of malignant thymoma.

What is malignant thymoma?

Malignant thymoma is a cancer of the thymus, a gland below the breastbone that is involved in producing infection-fighting cells. Malignant thymoma is most often seen in middle-aged or older people. There may be no symptoms, but some patients have cough, chest pain and difficulty breathing. Up to about half of all patients with malignant thymoma also have myasthenia gravis, a disease causing muscle weakness.

Malignant thymoma is a long-term debilitating and potentially life-threatening illness that is associated with poor long-term survival.

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

At the time of designation, malignant thymoma affected approximately 0.25 in 10,000 people in the European Union (EU). This was equivalent to a total of around 12,000 people*, and is below 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).

*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. This represents a population of 509,000,000 (Eurostat 2013).



What treatments are available?

At the time of designation, no satisfactory treatment was authorised in the EU for patients affected by the condition. Treatments for malignant thymoma included surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines to treat cancer).

How is this medicine expected to work?

Belinostat is an 'HDAC inhibitor' medicine. This means that it blocks enzymes called histone deacetylases (HDACs), which are involved in turning genes 'on' and 'off' within cells. By blocking HDAC enzymes, belinostat is expected to 'switch on' the genes that suppress the division and growth of the tumour cells in malignant thymoma. This is expected to lead to a reduction in the growth and spread of the cancer cells.

What is the stage of development of this medicine?

The effects of belinostat have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with belinostat in patients with malignant thymoma was ongoing.

At the time of submission, belinostat was not authorised anywhere in the EU for malignant thymoma 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 13 June 2013 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:

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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	Belinostat	Treatment of malignant thymoma
Bulgarian	БелиностаТ	Лечение на злокачвени тимомИ
Czech	Belinostat	Léčba maligního thymomu
Croatian	Belinostat	Liječenje malignog timoma
Danish	Belinostat	Behandling af malignt thymom
Dutch	Belinostat	Behandeling van maligne thymoma
Estonian	Belinostaat	Malignsete tümoomide ravi
Finnish	Belinostaatti	Pahanlaatuisten thymoomien hoito
French	Belinostat	Traitement du thymome malin
German	Belinostat	Behandlung von malignen Thymomen
Greek	Μπελινοστάτη	Θεραπεία κακοήθους θυμώματος
Hungarian	Belinosztat	Malignus thymoma kezelése
Italian	Belinostat	Trattamento del timoma maligno
Latvian	Belinostats	Malignas timomas ārstēšana
Lithuanian	Belinostatas	Piktybinės timomos gydymas
Maltese	Belinostat	Kura tat-timoma malinna
Polish	Belinostat	Leczenie grasiczaka złośliwego
Portuguese	Belinostato	Tratamento de timoma maligno
Romanian	Belinostat	Tratamentul timomului malign
Slovak	Belinostat	Liečba malígneho thymómu
Slovenian	Belinostat	Zdravljenje malignega limfoma
Spanish	Belinostat	Tratamiento del timoma maligno
Swedish	Belinostat	Behandling av maligna thymom
Norwegian	Belinostat	Behandling av malignt thymom
Icelandic	Belínóstat	Meðferð á illkynja thýmómi

¹ At the time of designation