



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

14 November 2012  
EMA/COMP/613019/2012  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Belinostat for the treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)

On 10 October 2012, orphan designation (EU/3/12/1055) was granted by the European Commission to Topotarget A/S, Denmark, for belinostat for the treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated).

### What is peripheral T-cell lymphoma?

Peripheral T-cell lymphoma is a cancer of the lymphatic system, a network of vessels that transport fluid from tissues through the lymph nodes and into the bloodstream. In peripheral T-cell lymphoma there is uncontrolled growth of T lymphocytes (T cells), a type of white blood cell found in the lymphatic system. Different types of peripheral T-cell lymphoma have been identified and categorised as nodal, other extranodal and leukaemic/disseminated.

The symptoms of the disease vary according to the type of lymphoma, but the first sign is usually a lump in the neck, under the arm or in the groin area, which is caused by an enlarged lymph node. The lymphoma may also affect other organs in the body such as the bone marrow, liver and the skin.

Peripheral T-cell lymphoma is a long-term debilitating and life-threatening condition because in most cases the disease does not respond well to therapy and comes back within one year after initial treatment and is associated with poor overall survival.

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

At the time of designation, peripheral T-cell lymphoma affected less than 1 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of fewer than 51,000 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 506,300,000 (Eurostat 2011).



## **What treatments are available?**

At the time of designation, there were no specific treatments for peripheral T-cell lymphoma, but the disease was treated in the same way as the broader class of lymphomas known as non-Hodgkin's lymphomas, for which several medicines were authorised in the EU. The main treatment was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that belinostat might be of significant benefit for patients with peripheral T-cell lymphoma because it works in a different way to existing treatments and early studies show increased anticancer activity when used in combination with currently available treatments. 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?**

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 peripheral T-cell lymphoma. This is expected to lead to a reduction in the growth and division 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, clinical trials with the medicine in patients with peripheral T-cell lymphoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for peripheral T-cell lymphoma. Orphan designation had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 September 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:

Topotarget A/S  
Fruebjergvej 3  
DK-2100 Copenhagen  
Denmark  
Telephone: +45 39 17 8392  
Telefax: +45 39 17 94 92  
E-mail: [enquiries@topotarget.com](mailto:enquiries@topotarget.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<sup>1</sup>, Norwegian and Icelandic

| Language   | Active ingredient | Indication  |
|------------|-------------------|---|
| English    | Belinostat        | Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)                  |
| Bulgarian  | Белиностат        | Лечение на периферен Т-клетъчен лимфом (нодален, екстранодален и левкемизирал/дисеминиран)                    |
| Czech      | Belinostat        | Léčba periferních T- lymfomů (nodální, extranodální a leukemické/disseminované)                               |
| Danish     | Belinostat        | Behandling af perifer T-celle lymfom (nodal, andre extranodale og leukæmiske/disseminerede)                   |
| Dutch      | Belinostat        | Behandeling van perifere T-cel lymfomen (nodale, andere extranodale en leukemische/uitgezaaide)               |
| Estonian   | Belinostaat       | Perifeerse T-rakulise lümfoomi (nodulaarne, teised ekstrasnodulaarsed ja leukeemilised/dissemineerunud) ravi  |
| Finnish    | Belinostaatti     | Perifeerisen T-solulymfooman hoito (nodaalinen, muu ekstrasnodaalinen ja leukeeminen/ disseminoitunut)        |
| French     | Belinostat        | Traitement du lymphome périphérique à cellules T (nodulaire, autre extra nodulaire et leucémique/disséminé)   |
| German     | Belinostat        | Behandlung des peripheren T-Zell-Lymphoms (nodulär, extranodulär und leukämisch/disseminiert)                 |
| Greek      | Μπελινοστάτη      | Θεραπεία του λεμφώματος περιφερικών κυττάρων Τ (λεμφαδενικό, άλλο εκτός λεμφαδένων και λευχαιμικό/ διάσπαρτο) |
| Hungarian  | Belinostat        | Perifériás T-sejtes lymphoma (nodalis, egyéb extranodalis és leukémiás/disszeminált) kezelése                 |
| Italian    | Belinostat        | Trattamento del linfoma periferico a cellule T (nodale, altre forme extranodali e leucemico/disseminato)      |
| Latvian    | Belinostats       | Perifēriskās T-šūnu limfomas (nodulāras, citas ekstrasnodulāras un leukēmiskas / diseminētas) ārstēšana       |
| Lithuanian | Belinostatas      | Periferinės T-ląstelių limfomos (mazginės, kitos ne mazginės ir leukeminės/difuzinės) gydymas                 |
| Maltese    | Belinostat        | Kura tal-linfoma taċ-ċelloli T periferali (fin-nodi, oħrajn barra n-nodi u lewkemiċi/imxerrdin)               |
| Polish     | Belinostat        | Leczenie obwodowego chłoniaka T-komórkowego (węzłowy, inny pozawęzłowy i białaczkopodobny/rozsiany)           |
| Portuguese | Belinostato       | Tratamento do linfoma periférico das células T (nodulares, outros extra nodulares e leucémicos/disseminados)  |
| Romanian   | Belinostat        | Tratamentul limfomului periferic cu celule T (ganglionar, extraganglionar și leucemic/diseminat)              |
| Slovak     | Belinostat        | Liečba periférneho T-bunkového lymfómu (nodálneho, iného extranodálneho a leukemického/disseminovaného)       |
| Slovenian  | Belinostat        | Zdravljenje perifernega limfoma celic T (nodalni, ekstrasnodalni, levkemični/diseminirani)                    |

<sup>1</sup> At the time of designation

| Language  | Active ingredient | Indication  |
|-----------|-------------------|---|
| Spanish   | Belinostat        | Tratamiento del linfoma periférico de células T (ganglionar, otros extraganglionares, leucémico/diseminado) |
| Swedish   | Belinostat        | Behandling av perifert T-cellslymfom (nodal, andra extranodala och leukemisk/spridd)                        |
| Norwegian | Belinostat        | Behandling av perifert T-celle-lymfom (nodalt, annet ekstranodalt og leukemisk/disseminert)                 |
| Icelandic | Belínóstat        | Meðferð við útlægu T-eitilfrumukrabbameini (í eitlum, utan þeirra og hvítblæðis/dreift)                     |