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EMA/COMP/286087/2007 Rev.2  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Panobinostat lactate for the treatment of cutaneous T-cell lymphoma

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|---|-----------------|
| First publication   | 21 January 2008 |
| Rev.1: withdrawal from the Community Register   | 8 November 2011 |
| Rev.2: sponsor's change of address  | 5 February 2015 |
| <b>Disclaimer</b><br>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***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in August 2011 on request of the Sponsor.***

On 2 August 2007, orphan designation (EU/3/07/464) was granted by the European Commission to Novartis Europharm Limited, UK, for panobinostat lactate for the treatment of cutaneous T cell lymphoma.

### What is cutaneous T cell lymphoma?

Cutaneous T cell lymphoma (CTCL) is a cancer of the lymphatic system. The lymphatic system is part of the body's immune system and helps fight infections. It is a complex system made up of organs such as bone marrow, thymus (a gland behind the breast bone), spleen (an organ in the abdomen near the stomach), and the lymph nodes (or lymph glands, located throughout the body), which are connected by a network of tiny lymphatic vessels. In addition, there are isolated lymphatic cells in most tissues of the body, including the skin. The main cells of the lymphatic system are the lymphocytes that belong to the group of white blood cells. There are two main types of lymphocytes: B-lymphocytes (B cells) and T-lymphocytes (T cells). Cutaneous T-cell lymphoma is characterised by uncontrolled growth of the T-lymphocytes and mainly affects the skin. This condition most often occurs in people aged between 40 and 60 years. Although in many cases the condition is chronic and survival for more than 10-20 years is common, cutaneous T-cell lymphoma can be a serious and life-threatening condition.



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

At the time of designation, cutaneous T cell lymphoma affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 130,000 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).

## **What treatments are available?**

Current treatment for cutaneous T-cell lymphoma can be divided into local and systemic (given to the whole body) treatments. Local treatments include medicines applied to the skin, therapies using light of a particular wavelength (ultraviolet light) and x-rays. At the time of submission of the application for orphan drug designation, systemic treatments included medicines such as glucocorticoids (a group of medicines that are similar to cortisone), cytotoxic agents (medicines that kill cells), interferon-alfa (a compound that can help the immune system to fight against the cancer cells) and photopheresis (blood is temporarily removed from the body and exposed to ultraviolet light after being treated with a sensitizing compound). Several products were authorised for the treatment of cutaneous T cell lymphoma within the Community at the time of submission of the application for orphan drug designation.

Panobinostat lactate might be of potential significant benefit for the treatment of cutaneous T-cell lymphoma because it is expected to act in a different way to other available medicines. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status of panobinostat lactate.

## **How is this medicine expected to work?**

Panobinostat lactate blocks the action of a group of enzymes in cells. Enzymes are protein molecules that facilitate chemical reactions in cells. The enzymes blocked by panobinostat are called histone deacetylases, and they are involved in cell growth and replication. By blocking these enzymes, panobinostat lactate is expected to reduce or arrest the growth of several types of cancers, including cutaneous T-cell lymphoma.

## **What is the stage of development of this medicine?**

At the time of submission of the application for orphan designation, the effects of panobinostat lactate had been evaluated in experimental models, and clinical trials in patients with cutaneous T-cell lymphomas were ongoing.

Panobinostat lactate was not authorised anywhere in the world for cutaneous T-cell lymphoma, or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 27 June 2007 recommending the granting of this designation.

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\*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 500,300,000 (Eurostat 2007).

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  
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Camberley GU16 7SR  
United Kingdom  
Tel. +41 61 324 11 11 (Switzerland)  
E-mail: [orphan.enquiries@novartis.com](mailto: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<sup>1</sup>, Norwegian and Icelandic

| Language   | Active Ingredient        | Indication                                       |
|------------|--------------------------|--|
| English    | Panobinostat lactate     | Treatment of cutaneous T-cell lymphoma           |
| Bulgarian  | Панобиностат лактат      | Лечение на кожен Т-клетъчен лимфом               |
| Czech      | Panobinostat laktát      | Léčba kožního T-lymfomu                          |
| Danish     | Panobinostatlaktat       | Behandling af kutant T-celle-lymfom              |
| Dutch      | Panobinostat lactaat     | Behandeling van cutaan T-cel-lymfoom             |
| Estonian   | Panobinostaat-laktaat    | Kutaanse T-rakulise lümfoomi ravi                |
| Finnish    | Panobinostaattilaktaatti | Ihon T-solulymfooman hoito                       |
| French     | Lactate de panobinostat  | Traitement des lymphomes cutanés à cellules T    |
| German     | Panobinostat Laktat      | Behandlung von kutanem T-Zell- Lymphom           |
| Greek      | Γαλακτική Πανομπινοστάτη | Θεραπεία του δερματικού λεμφώματος Τα κυττάρων   |
| Hungarian  | Panobinosztát laktát     | T-sejtes cutan lymphoma kezelése                 |
| Italian    | Panobinostat lattato     | Trattamento del linfoma cutaneo a cellule T      |
| Latvian    | Panobinostata laktāts    | Ādas T-šūnu limfomas ārstēšana                   |
| Lithuanian | Panobinostato laktatas   | Odos T ląstelių limfomos gydymas                 |
| Maltese    | Panobinostat lactate     | Kura tal-linfoma taċ-ċelluli tat-tip T tal-ġilda |
| Polish     | Mleczan panobinostatu    | Leczenie chłoniaka skórniego T-komórkowego       |
| Portuguese | Lactato de panobinostat  | Tratamento do linfoma cutâneo de células T       |
| Romanian   | Lactat de panobinostat   | Tratamentul limfomului cutanat cu celule T       |
| Slovak     | Panobinostati lactas     | Liečba kutánneho T-bunkového lymfómu             |
| Slovenian  | Panobinostat laktat      | Zdravljenje kožnega T-celičnega limfoma          |
| Spanish    | Lactato de panobinostato | Tratamiento del linfoma cutáneo de células T     |
| Swedish    | Panobinostat laktat      | Behandling av kutant T-cellslymfom               |
| Norwegian  | Panobinostatlaktat       | Behandling av kutant T-cellelymfom               |
| Icelandic  | Panóbínóstat laktat      | Meðferð T-eitilfrumukrabbameins í húð            |

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