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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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

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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 6 December 2012, orphan designation (EU/3/12/1074) was granted by the European Commission to Takeda Global Research and Development Centre (Europe) Ltd, United Kingdom, for alisertib for the treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated).

In November 2013, Takeda Global Research and Development Centre (Europe) Ltd changed name to Takeda Development Centre Europe Ltd.

What is peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)?

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 are classified 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, usually comes back within one year and is associated with poor overall survival.

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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 was 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).

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 alisertib 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 showed complete and partial responses in patients with peripheral T-cell lymphoma that is aggressive, has come back after or does not respond to other 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?

Alisertib is an Aurora A kinase inhibitor. This means that it works by blocking the activity of an enzyme known as Aurora A kinase, which plays an important role in the control of cell division. Aurora A kinase is found in high amounts in cancer cells. By blocking this enzyme, alisertib is expected to stop the cancer cells from dividing, eventually killing them. This is expected to slow down or stop the growth of the cancer.

What is the stage of development of this medicine?

The effects of alisertib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with alisertib in patients with peripheral T-cell lymphoma were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 November 2012 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 509,000,000 (Eurostat 2012).

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 languages[1], Norwegian and Icelandic

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

[1] At the time of designation

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