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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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

On 20 March 2007, orphan designation (EU/3/07/438) was granted by the European Commission to Serono Europe Limited, United Kingdom, for zanolimumab for the the treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated).

The sponsorship was transferred to Genmab A/S, Denmark, in March 2008 and to TenX Biopharma Ltd, United Kingdom, in September 2010.

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

Peripheral T-cell lymphomas belong to the group of non-Hodgkin's lymphomas, which are cancers originating from the lymphatic system. The lymphatic system is part of the body's immune system, and helps fighting infections. It is a complex system made up of organs such as the bone marrow, the thymus (a gland behind the breast bone), the 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. There are two main types of cells, which make up the lymphatic tissue. These cells are called lymphocytes and belong to the broader group of white blood cells. The two types are called B lymphocytes (B cells) and T lymphocytes (T cells). Most lymphocytes start growing in the bone marrow. The T cells go from the bone marrow to the thymus where they continue to mature. Normally, the lymphatic cells grow in a controlled manner. Peripheral T-cell lymphoma is caused by the uncontrolled growth of T-lymphocytes in different stages of maturity. Different types of peripheral T-cell lymphoma have been identified and categorised (nodal, other extranodal and leukaemic/disseminated). Patients most often present with generalised lymph node enlargement, liver enlargement and bone marrow involvement. Peripheral T-cell lymphoma is a serious and life-threatening condition.

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

At the time of designation, peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated) affected less than 1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 46,000 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?

There are currently several medicinal products authorised in the Community for treatment of non-Hodgkin lymphoma. The choice of treatment depends in particular on the extension of the disease as well as on the responses to therapies previously prescribed. Although chemotherapy (using medicines to kill cancer cells) is the current standard of care for peripheral T-cell lymphoma, most tumours will come back and then more intensive regimens of several chemotherapeutic agents are given.

Zanolimumab might be of potential significant benefit for the treatment of peripheral T-cell lymphoma because it is expected to act in a different way from 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.

How is this medicine expected to work?

Most of the cells that grow in peripheral T-cell lymphomas have a protein on the outer surface of the cell, called CD4; therefore, these cells are known as CD4 positive cells. Immunoglobulins are proteins present in the blood and are involved in the immune response against foreign substances (antigens). The product is a human immunoglobulin (of the IgG1κ class) that has the capacity to bind the CD4 protein. By binding to the CD4 protein on the surface of the T-cells, the product induces the death of the CD4-positive cells. This is expected to result in destruction of the cells that form the lymphoma.

What is the stage of development of this medicine?

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

Zanolimumab was not authorised anywhere worldwide for peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated) 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 7 February 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 based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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

[1] At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Slovenian	Zanolimumab	Zdravljenje perifernega limfoma celic T (nodalni, ekstranodalni, levkemični/diseminirani)
Spanish	Zanolimumab	Tratamiento del linfoma periférico de células T (ganglionar, otros extraganglionares, leucémico/diseminado)
Swedish	Zanolimumab	Behandling av perifert T-cellslymfom (nodal, andra extranodala och leukemisk/spridd)
Norwegian	Zanolimumab	Behandling av perifert T-celle-lymfom (nodalt, annet ekstranodalt og leukemisk/disseminert)
Icelandic	Zanólímúmab	Meðferð við útlægu T-eitilfrumukrabbameini (í eitlum, utan þeirra og hvítblæðis/dreift)