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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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

On 11 January 2012, orphan designation (EU/3/11/943) was granted by the European Commission to Gregory Fryer Associates Ltd, United Kingdom, for mogamulizumab for the treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated).

The sponsorship was transferred to ProStrakan Limited, United Kingdom, in April 2012.

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, which appear in the blood circulating in peripheral parts of the body. 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 serious and life-threatening condition because in most cases the disease 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 approximately 0.5 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 25,000 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).

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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 mogamulizumab 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 that it may improve the treatment of patients whose lymphoma cells carry a receptor called CCR4 on their surface. 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?

Mogamulizumab is a monoclonal antibody, an antibody (a type of protein) that has been designed to recognise and attach to a specific structure (called an antigen) that is found in the body. It is expected to attach to the CCR4 receptor which is found on the surface of T cells including the cancerous T cells seen in patients with peripheral T-cell lymphoma. By attaching to CCR4, mogamulizumab stimulates the body's immune system to attack the cancerous cells, helping to control the disease.

What is the stage of development of this medicine?

The effects of mogamulizumab have been evaluated in experimental models.

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

At the time of submission, mogamulizumab was not authorised anywhere in the EU for peripheral T-cell lymphoma. Orphan designation of mogamulizumab 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 9 November 2011 recommending the granting of this designation.

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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	Mogamulizumab	Treatment of peripheral T-cell lymphoma (nodal, other extranodal and leukaemic/disseminated)
Bulgarian	Могамулизумаб	Лечение на периферен Т-клетъчен лимфом (нодален, екстранодален и левкемизирал/дисеминиран)
Czech	Mogamulizumab	Léčba periferních T- lymfomů (nodální, extranodální a leukemické/diseminované)
Danish	Mogamulizumab	Behandling af perifer T-celle lymfom (nodal, andre extranodale og leukæmiske/disseminerede)
Dutch	Mogamulizumab	Behandeling van perifere T-cel lymfomen (nodale, andere extranodale en leukemische/uitgezaaïde)
Estonian	Mogamulisumab	Perifeerse T-rakulise lümfoomi (nodulaarne, teised ekstranodulaarsed ja leukeemilised/dissemineerunud) ravi
Finnish	Mogamulitsumabi	Perifeerisen T-solulymfooman hoito (nodaalinen, muu ekstranodaalinen ja leukeeminen/ disseminoitunut)
French	Mogamulizumab	Traitement du lymphome périphérique à cellules T (nodulaire, autre extra nodulaire et leucémique/disséminé)
German	Mogamulizumab	Behandlung des peripheren T-Zell-Lymphoms (nodulär, extranodulär und leukämisch/disseminiert)
Greek	Μογκαμουλιζουμάμπη	Θεραπεία του λεμφώματος περιφερικών κυττάρων Τ (λεμφαδενικό, άλλο εκτός λεμφαδένων και λευχαιμικό/ διάσπαρτο)
Hungarian	Mogamulizumab	Perifériás T-sejtes lymphoma (nodalis, egyéb extranodalis és leukémiás/disszeminált) kezelése
Italian	Mogamulizumab	Trattamento del linfoma periferico a cellule T (nodale, altre forme extranodali e leucemico/disseminato)
Latvian	Mogamulizumabs	Perifēriskās T-šūnu limfomas (nodulāras, citas ekstranodulāras un leikēmiskas / diseminētas) ārstēšana
Lithuanian	Mogamulizumabas	Periferinės T-ląstelių limfomos (mazginės, kitos ne mazginės ir leukeminės/difuzinės) gydymas
Maltese	Mogamulizumab	Kura tal-limfoma taċ-ċelloli T periferali (fin-nodi, oħrajn barra n-nodi u lewkemiċi/imxerrdin)
Polish	Mogamulizumab	Leczenie obwodowego chłoniaka T-komórkowego (węzłowy, inny pozawęzłowy i białaczkopodobny/rozsiany)
Portuguese	Mogamulizumab	Tratamento do linfoma periférico das células T (nodulares, outros extra nodulares e leucémicos/disseminados)

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Mogamulizumab	Tratamentul limfomului periferic cu celule T (ganglionar, extraganglionar şi leucemic/diseminat)
Slovak	Mogamulizumab	Liečba periférneho T-bunkového lymfómu (nodálneho, iného extranodálneho a leukemického/diseminovaného)
Slovenian	Mogamulizumab	Zdravljenje perifernega limfoma celic T (nodalni, ekstranodalni, levkemični/diseminirani)
Spanish	Mogamulizumab	Tratamiento del linfoma periférico de células T (ganglionar, otros extraganglionares, leucémico/diseminado)
Swedish	Mogamulizumab	Behandling av perifert T-cellslymfom (nodal, andra extranodala och leukemisk/spridd)
Norwegian	Mogamulizumab	Behandling av perifert T-celle-lymfom (nodalt, annet ekstranodalt og leukemisk/disseminert)
Icelandic	Mógamúlízúmab	Meðferð við útlægu T-eitilfrumukrabbameini (í eitlum, utan þeirra og hvítblæðis/dreift)