EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Obinutuzumab for the treatment of marginal zone lymphoma

On 19 June 2015, orphan designation (EU/3/15/1505) was granted by the European Commission to Roche Registration Limited, United Kingdom, for obinutuzumab for the treatment of marginal zone lymphoma.

What is marginal zone lymphoma?

Marginal zone lymphoma is a cancer of a type of white blood cell called B lymphocytes or B cells. In marginal zone lymphoma, abnormal B cells multiply too quickly and live for too long. The abnormal B cells affect various organs. Patients usually have fever, weight loss, tiredness and night sweats.

Marginal zone lymphoma is a life-threatening and long-term debilitating disease due to its effects on the spleen, lymph nodes and bone marrow, as well as the increased risk of infection.

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

At the time of designation, marginal zone lymphoma affected approximately 0.9 in 10,000 people in the European Union (EU). This was equivalent to a total of around 46,000 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, the main treatments for marginal zone lymphoma available in the EU included immunotherapy (using the body's own immune system to kill cancer cells) with the medicine rituximab, chemotherapy (cancer medicines), radiotherapy (treatment with radiation) and surgery to remove affected lymph nodes. In some patients, marginal zone lymphoma affecting the stomach is associated with infection by the bacterium *Helicobacter pylori*, and treatment with antibiotics was used to resolve the infection.

[*]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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with marginal zone lymphoma because early studies show that patients whose disease has come back after treatment might respond to treatment with obinutuzumab. 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?

Obinutuzumab is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a specific structure (called an antigen) that is found on certain cells in the body. Obinutuzumab has been designed to attach to a protein called CD20, which is present on the surface of all B cells, including the large number of cancerous B cells that are present in the lymph nodes of patients with marginal zone lymphoma. When obinutuzumab attaches to the CD20, it is expected to cause the death of the cancerous B cells, slowing down the growth of the cancer.

Obinutuzumab is currently authorised in the EU as Gazyvaro for use in combination with chlorambucil (another cancer medicine) to treat patients with previously untreated chronic lymphocytic leukaemia.

What is the stage of development of this medicine?

The effects of obinutuzumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with obinutuzumab in patients with marginal zone lymphoma were ongoing.

At the time of submission, obinutuzumab was not authorised anywhere in the EU for marginal zone lymphoma. Orphan designation of obinutuzumab has been granted in the United States for splenic marginal zone lymphoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 May 2015 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:

Roche Registration Limited
6 Falcon Way
Shire Park
Welwyn Garden City
AL7 1TW
United Kingdom
Tel. +44 (0)1707 362 840
Fax +44 (0)1707 377 838
E-mail: info.orphan@roche.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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Obinutuzumab	Treatment of marginal zone lymphoma
Bulgarian	Обинутузумаб	Лечение на маргинално зонален лимфом
Croatian	Obinutuzumab	Liječenje limfoma marginalne zone
Czech	Obinutuzumab	Léčba lymfomu z marginální zóny
Danish	Obinutuzumab	Behandling af marginalzonelymfom
Dutch	Obinutuzumab	Behandeling van marginale zone lymfoom
Estonian	Obinutuzumab	Marginaaltsooni lümfoomi ravi
Finnish	Obinututsumabi	Marginaalivyöhykkeen lymfooman hoito
French	Obinutuzumab	Traitement du lymphome de la zone marginale
German	Obinutuzumab	Behandlung des Marginalzonenlymphoms
Greek	Ομπινουτουζουμάμπη	Θεραπεία του λεμφώματος μεθοριακής ζώνης
Hungarian	Obinutuzumab	Marginális zóna lymphoma kezelése
Italian	Obinutuzumab	Trattamento del linfoma della zona marginale
Latvian	Obinutuzumabs	Marginālo zonu limfomas ārstēšana
Lithuanian	Obinutuzumabas	Marginalinės zonos limfomos gydymas
Maltese	Obinutuzumab	Kura ta' limfoma taż-żona marġinali
Polish	Obinutuzumab	Leczenie chłoniaka strefy brzeżnej
Portuguese	Obinutuzumab	Tratamento do linfoma da zona marginal
Romanian	Obinutuzumab	Tratamentul limfomului de zonă marginală
Slovak	Obinutuzumab	Liečba lymfómu z marginálnej zóny
Slovenian	Obinutuzumab	Zdravljenje limfoma marginalne cone
Spanish	Obinutuzumab	Tratamiento del linfoma de la zona marginal
Swedish	Obinutuzumab	Behandling av marginalzonslymfom
Norwegian	Obinutuzumab	Behandling av marginalsonelymfom
Icelandic	Óbínútúzumab	Meðferð við jaðarsvæðiseitilkrabbameini

[1] At the time of designation