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EMA/COMP/346519/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Obinutuzumab for the treatment of follicular lymphoma

On 19 June 2015, orphan designation (EU/3/15/1504) was granted by the European Commission to Roche Registration Limited, United Kingdom, for obinutuzumab for the treatment of follicular lymphoma.

What is follicular lymphoma?

Follicular lymphoma is a cancer of a type of white blood cell called B lymphocytes or B cells. In follicular lymphoma, the B cells multiply too quickly and live for too long, so there are too many of them in the lymph nodes. The first sign of the disease is usually a lump in the neck, under the arm or in the groin area, caused by an enlarged lymph node. Patients may also have fever, weight loss, tiredness and night sweats.

Follicular lymphoma is usually diagnosed in people aged over 50 years. It is a life-threatening and long-term debilitating disease due to organ damage and the cancer coming back.

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

At the time of designation, follicular lymphoma affected approximately 2.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 123,000 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 follicular lymphoma available in the EU included chemotherapy (medicines to treat cancer) combined with immunotherapy (medicines that stimulate the body's own immune system to kill the cancer cells). The medicines bendamustine, ibritumomab

*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).

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tiuxetan, interferon alfa 2b and rituximab were specifically authorised for the treatment of follicular lymphoma.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with follicular 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 follicular lymphoma. When obinutuzumab attaches to 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 follicular lymphoma were ongoing.

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

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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	Obinutuzumab	Treatment of follicular lymphoma
Bulgarian	Обинутузумаб	Лечение на фоликуларен лимфом
Croatian	Obinutuzumab	Liječenje folikularnog limfoma
Czech	Obinutuzumab	Léčba folikulárního lymfomu
Danish	Obinutuzumab	Behandling af follikulært lymfom
Dutch	Obinutuzumab	Behandeling van folliculair lymfoom
Estonian	Obinutuzumab	Follikulaarse lümfoomi ravi
Finnish	Obinututsumabi	Follikulaarisen lymfooman hoito
French	Obinutuzumab	Traitement des lymphomes folliculaires
German	Obinutuzumab	Behandlung des follikulären Lymphoms
Greek	Ομπινουτουζουμάμπη	θεραπεία του θηλακιώδους λεμφώματος
Hungarian	Obinutuzumab	Follicularis lymphoma kezelése
Italian	Obinutuzumab	Trattamento del linfoma follicolare
Latvian	Obinutuzumabs	Folikulārās limfomas ārstēšana
Lithuanian	Obinutuzumabas	Folikulinės limfomos gydymas
Maltese	Obinutuzumab	Kura tal-limfoma follikulari
Polish	Obinutuzumab	Leczenie chłoniaków grudkowych
Portuguese	Obinutuzumab	Tratamento do linfoma folicular
Romanian	Obinutuzumab	Tratamentul limfomului folicular
Slovak	Obinutuzumab	Liečba folikulárneho lymfómu
Slovenian	Obinutuzumab	Zdravljenje folikularnega limfoma
Spanish	Obinutuzumab	Tratamiento del linfoma folicular
Swedish	Obinutuzumab	Behandling av follikulärt lymfom
Norwegian	Obinutuzumab	Behandling av follikulært lymfom
Icelandic	Óbínútúzumab	Meðferð á follicular eitilfrumukrabbameini

[1] At the time of designation