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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Obinutuzumab for the treatment of chronic lymphocytic leukaemia

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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 10 October 2012, orphan designation (EU/3/12/1054) was granted by the European Commission to Roche Registration Limited, United Kingdom, for obinutuzumab for the treatment of chronic lymphocytic leukaemia.

What is chronic lymphocytic leukaemia?

Chronic lymphocytic leukaemia (CLL) is cancer of a type of white blood cell called B-lymphocytes. In this disease, the lymphocytes multiply too quickly and live for too long, so that there are too many of them circulating in the blood. The cancerous lymphocytes look normal, but they are not fully developed and do not work properly. Over a period of time, the abnormal cells replace the normal white cells, red cells and platelets (components that help the blood to clot) in the bone marrow (the spongy tissue inside the large bones in the body).

CLL is the most common type of leukaemia and mainly affects older people. It is rare in people under the age of 40 years. CLL is a long-term debilitating and life-threatening disease because some patients develop severe infections.

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

At the time of designation, CLL affected approximately 3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 153,000 people, and is below the ceiling for orphan

*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).

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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?

Treatment for CLL is complex and depends on a number of factors, including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. Patients whose CLL is not causing any symptoms or is only getting worse very slowly may not need treatment. Treatment for CLL is only started if symptoms become troublesome. At the time of designation, the main treatment for CLL was chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with CLL because early studies suggest that it might improve the outcome of patients whose disease has come back after treatment. 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 target an antigen called CD20, which is present on the surface of all B-lymphocytes. When obinutuzumab attaches to the CD20, this is expected to cause cell death of the B-lymphocytes, improving the symptoms of the disease.

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 CLL were ongoing.

At the time of submission, obinutuzumab was not authorised anywhere in the EU for CLL. Orphan designation of obinutuzumab had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 September 2012 recommending the granting of this designation.

Update: obinutuzumab (Gazyvaro) has been authorised in the EU since 23 July 2014. Gazyvaro in combination with chlorambucil is indicated for the treatment of adult patients with previously untreated chronic lymphocytic leukaemia (CLL) and with comorbidities making them unsuitable for full-dose fludarabine based therapy.

More information on Gazyvaro can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

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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 chronic lymphocytic leukaemia
Bulgarian	Обинутузумаб	Лечение на хронична лимфоцитна левкемия
Czech	Obinutuzumab	Léčba chronické lymfatické leukémie
Danish	Obinutuzumab	Behandling af kronisk lymfocytær leukæmi
Dutch	Obinutuzumab	Behandeling van chronische lymfocytaire leukemie
Estonian	Obinutuzumab	Kroonilise lümfoidleukeemia ravi
Finnish	Obinututsumabi	Kroonisen lymfosyyttileukemian hoito
French	Obinutuzumab	Traitement de la leucémie lymphoïde chronique
German	Obinutuzumab	Behandlung der chronisch-lymphatischen Leukämie
Greek	Ομπινουτουζουμάμπη	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας
Hungarian	Obinutuzumab	Krónikus lymphoid leukémia kezelése
Italian	Obinutuzumab	Trattamento della leucemia linfocitica cronica
Latvian	Obinutuzumabs	Hroniskas limfocitiskās leikēmijas ārstēšana
Lithuanian	Obinutuzumabas	Lėtinės limfocitinės leukemijos gydymas
Maltese	Obinutuzumab	Kura tal-lewkimja limfoċitika kronika
Polish	Obinutuzumab	Leczenie przewlekłej białaczki limfatycznej
Portuguese	Obinutuzumab	Tratamento da leucemia linfocítica crónica
Romanian	Obinutuzumab	Tratamentul leucemiei limfoide cronice
Slovak	Obinutuzumab	Liečba chronickej lymfocytovej leukémie
Slovenian	Obinutuzumab	Zdravljenje kronične limfatske levkemije
Spanish	Obinutuzumab	Tratamiento de la leucemia linfocítica crónica
Swedish	Obinutuzumab	Behandling av kronisk lymfatisk leukemi
Norwegian	Obinutuzumab	Behandling av kronisk lymfatisk leukemi
Icelandic	Óbínútúzumab	Meðferð á langvinnu eitilfrumuhvítblæði

[1] At the time of designation