23 September 2014
EMA/COMP/452142/2014
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Obinutuzumab for the treatment of diffuse large B-cell lymphoma

On 22 August 2014, orphan designation (EU/3/14/1325) was granted by the European Commission to Roche Registration Limited, United Kingdom, for obinutuzumab for the treatment of diffuse large B-cell lymphoma.

### What is diffuse large B-cell lymphoma?

Diffuse large B-cell lymphoma is a cancer that affects a type of white blood cell called B lymphocytes, or B cells. In patients with this cancer, 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, which is caused by an enlarged lymph node. Patients with diffuse large B-cell lymphoma may also have fever, tiredness, night sweats or weight loss that have no obvious cause.

Although some people with diffuse large B-cell lymphoma can be cured, it remains a serious and life-threatening disease, particularly when the disease is diagnosed late or has come back after initial treatment.

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

At the time of designation, diffuse large B-cell lymphoma affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 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, several medicines were authorised for the treatment of diffuse large B-cell lymphoma in the EU. The main treatment was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation). Autologous haematopoietic (blood) stem cell

*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 511,100,000 (Eurostat 2014).

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

transplantation was also used in patients at risk of the disease coming back after treatment. This is a complex procedure where patients receive their own stem cells to help restore the bone marrow.

The sponsor has provided sufficient information to show that obinutuzumab might be of significant benefit for patients with diffuse large B-cell lymphoma because early studies show that it might improve the outcome of patients whose disease does not respond to or 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 cells. When obinutuzumab attaches to the CD20, this is expected to cause death of the B cells, 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 diffuse large B-cell lymphoma were ongoing.

At the time of submission, obinutuzumab was not authorised anywhere in the EU for diffuse large B-cell lymphoma. Orphan designation of obinutuzumab had been granted in the United States for this condition, and in the EU and the US for the treatment of chronic lymphocytic leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 July 2014 recommending the granting of this designation.

_____

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. +44 170 7362 840
Fax +44 170 7377 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 diffuse large B-cell lymphoma |
| Bulgarian | Обинутузумаб | Лечение на дифузен В-едроклетъчен лимфом |
| Croatian | Obinutuzumab | Liječenje difuznog limfoma velikih B-stanica |
| Czech | Obinutuzumab | Léčba velkobuněčného difuzního B-lymfomu |
| Danish | Obinutuzumab | Behandling af diffust storcellet B-celle lymfom |
| Dutch | Obinutuzumab | Behandeling van diffuus grootcellig B-cel-lymfoom |
| Estonian | Obinutuzumab | Diffuusse suure β-rakulise lümfoomi ravi |
| Finnish | Obinututsumabi | Diffuusin suurisoluisen B-solulymfooman hoito |
| French | Obinutuzumab | Traitement du lymphome diffus à grandes cellules B |
| German | Obinutuzumab | Behandlung des diffusen großzelligen B-Zell-Lymphoms |
| Greek | Ομπινουτουζουμάμπη | Θεραπεία του διάχυτου μεγαλοκυτταρικού λεμφώματος Β-κυττάρου (DLBCL) |
| Hungarian | Obinutuzumab | Diffúz nagy B-sejtes lymphoma kezelése |
| Italian | Obinutuzumab | Terapia del Linfoma non-Hodgkin diffuso a grandi cellule di tipo B (DLBCL) |
| Latvian | Obinutuzumabs | Difūzas lielo B šūnu limfomas ārstēšana |
| Lithuanian | Obinutuzumabas | Difuzinės stambiųjų B ląstelių limfomos gydymas |
| Maltese | Obinutuzumab | Kura tal-limfoma taċ-ċelluli tat-tip B kbar mxerrda |
| Polish | Obinutuzumab | Leczenie rozlanego chłoniaka z dużych limfocytów B |
| Portuguese | Obinutuzumab | Tratamento do linfoma difuso de grandes células B |
| Romanian | Obinutuzumab | Tratamentul limfomului difuz cu celule B mari |
| Slovak | Obinutuzumab | Liečba difúzneho veľkobunkového lymfómu z buniek B |
| Slovenian | Obinutuzumab | Zdravljenje razširjenega limfoma velikih B celic |
| Spanish | Obinutuzumab | Tratamiento del linfoma difuso de células B grandes |
| Swedish | Obinutuzumab | Behandling av diffusa storcelliga B-cells lymfom |
| Norwegian | Obinutuzumab | Behandling av diffust storcellet B-celle lymfom |
| Icelandic | Óbínútúzumab | Til meðferðar á dreifðu stórfrumu B frumu eitlakrabbameini |

[1] At the time of designation