



21 May 2015
EMA/COMP/480244/2008 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ofatumumab for the treatment of chronic lymphocytic leukaemia

First publication	1 April 2009
Rev.1: information about Marketing Authorisation	11 June 2010
Rev.2: transfer of sponsorship	21 May 2015
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 7 November 2008, orphan designation (EU/3/08/581) was granted by the European Commission to Glaxo Group Limited, United Kingdom, for ofatumumab for the treatment of chronic lymphocytic leukaemia.

The sponsorship was transferred to Novartis Europharm Limited, United Kingdom, in May 2015.

What is chronic lymphocytic leukaemia?

Chronic lymphocytic leukaemia is a disease in which cancer cells are found in the blood and the bone marrow. The bone marrow is the spongy tissue inside the large bones in the body. Normally, the bone marrow makes cells, called "blasts", which mature into several different types of blood cells with specific functions in the body. These include red cells, white cells and platelets. Red blood cells carry oxygen and other materials to all tissues of the body. White blood cells fight infection. Platelets support blood clotting. When leukaemia develops, the bone marrow produces large numbers of abnormal blood cells. Over a period of time these abnormal cells replace the normal white cells, red cells and platelets in the bone marrow, which reduces the number of normal cells in the blood and leads to anaemia, coagulation problems (bruising, haemorrhages) and repeated infections. There are several types of leukaemias. Chronic lymphocytic leukaemia is a cancer of a type of white blood cell called the B-lymphocyte. The lymphocytes multiply and live too long, so there are too many of them circulating in the blood. These leukaemic lymphocytes look normal, but they are not fully developed and do not work properly. Chronic lymphocytic leukaemia is the most common type of leukaemia; it mainly affects older people, being rare in people under the age of 40. Chronic lymphocytic leukaemia is chronically



debilitating and life-threatening, due to the severe prognosis and the poor long-term survival for high-risk patients.

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

At the time of designation, chronic lymphocytic leukaemia affected approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 176,000 people*, and is below the threshold 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?

Treatment for leukaemia is complex, and depends on a number of factors including the type of leukaemia, the extent of the disease and whether the leukaemia has been treated before. It also depends on the age, symptoms, and the general health of the patient. Some people with B-cell chronic lymphocytic leukaemia never have treatment, if their illness is not causing any symptoms and is progressing slowly. Treatment is often started only if and when the symptoms become troublesome. Current main treatment of chronic lymphocytic leukaemia is chemotherapy (using drugs to kill cancer cells). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation.

Ofatumumab could be of potential significant benefit for the treatment of chronic lymphocytic leukaemia, because it might act differently from other medicinal products. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Ofatumumab is a monoclonal antibody. A monoclonal antibody is an antibody (a type of protein) that has been designed to recognise and bind to a specific structure (called an antigen) that is found on certain cells in the body.

Ofatumumab has been designed to target an antigen called CD20, which is present on the surface of all B-lymphocytes. When ofatumumab binds to the antigen, this causes lysis (cell death). This is expected to help in chronic lymphocytic leukaemia by destroying the cancerous B-lymphocytes.

What is the stage of development of this medicine?

The effects of ofatumumab were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with chronic lymphocytic leukaemia were ongoing.

Ofatumumab was not authorised anywhere worldwide for chronic lymphocytic leukaemia or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 September 2008 recommending the granting of this designation.

*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 502,800,000 (Eurostat 2008).

Update: Ofatumumab (Arzerra) has been authorised in the EU since 19 April 2010 for the treatment of chronic lymphocytic leukaemia (CLL) in patients who are refractory to fludarabine and alemtuzumab.

More information on Arzerra 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](http://ema.europa.eu/Find_medicine/Human_medicines/European_Public_Assessment_Reports)

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 European Union) 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](http://orphanet.eu), a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- [European Organisation for Rare Diseases \(EURORDIS\)](http://eurordis.eu), 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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Ofatumumab	Treatment of chronic lymphocytic leukaemia
Bulgarian	Офатумумаб	Лечение на хронична лимфоцитна левкемия
Croatian	Ofatumumab	Liječenje kronične limfocitne leukemije
Czech	Ofatumumab	Léčba chronické lymfatické leukémie
Danish	Ofatumumab	Behandling af kronisk lymfocytær leukæmi
Dutch	Ofatumumab	Behandeling van chronische lymfocyttaire leukemie
Estonian	Ofatumumab	Kroonilise lümfoïdleukeemia ravi
Finnish	Ofatumumabi	Kroonisen lymfosyyttileukemian hoito
French	Ofatumumab	Traitement de la leucémie lymphoïde chronique
German	Ofatumumab	Behandlung der chronisch-lymphatischen Leukämie
Greek	Οφατουμουμάμπη	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας
Hungarian	Ofatumumab	Krónikus lymphoid leukémia kezelése
Italian	Ofatumumab	Trattamento della leucemia linfocitica cronica
Latvian	Ofatumumabs	Hroniskas limfocitiskās leikēmijas ārstēšana
Lithuanian	Ofatumumabas	Lėtinės limfocitinės leukemijos gydymas
Maltese	Ofatumumab	Kura tal-lewkimja limfoċitika kronika
Polish	Ofatumumab	Leczenie przewlekłej białaczki limfatycznej
Portuguese	Ofatumumab	Tratamento da leucémia linfocítica crónica
Romanian	Ofatumumab	Tratamentul leucemiei limfoide cronice
Slovak	Ofatumumab	Liečba chronickej lymfocytovej leukémie
Slovenian	Ofatumumab	Zdravljenje kronične limfatske levkemije
Spanish	Ofatumumab	Tratamiento de la leucemia linfocítica crónica
Swedish	Ofatumumab	Behandling av kronisk lymfatisk leukemi
Norwegian	Ofatumumab	Behandling av kronisk lymfatisk leukemi
Icelandic	Ofatúmúmab	Meðferð á langvinnu eitilfrumuhvítblæði

¹ At the time of transfer of sponsorship