



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

London, 8 December 2009
Doc.Ref.: EMEA/COMP/663075/2009

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of recombinant chimeric monoclonal antibody against CD20 for the treatment of chronic lymphocytic leukaemia

On 26 November 2009, orphan designation (EU/3/09/699) was granted by the European Commission to LFB-Biotechnologies, France, for recombinant chimeric monoclonal antibody against CD20 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 3.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of 177,000 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 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 recombinant chimeric monoclonal antibody against CD20 might be of significant benefit for patients with CLL because early studies in experimental models indicate that it might improve the treatment of patients with this condition. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

*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. This represents a population of 504,800,000 (Eurostat 2009).

How is this medicine expected to work?

Recombinant chimeric monoclonal antibody against CD20 is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a specific structure (an antigen) called CD20. This is a receptor that is found on the surface of all B-lymphocytes. When this medicine binds CD20, this is expected to cause cell death. This is expected to help in CLL by destroying the cancerous B-lymphocytes.

What is the stage of development of this medicine?

The effects of recombinant chimeric monoclonal antibody against CD20 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the designated product in patients with CLL were ongoing.

At the time of submission, recombinant chimeric monoclonal antibody against CD20 was not authorised anywhere in the EU for CLL or designated as orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2009 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 Community) 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:

LFB-Biotechnologies

59, rue de Trévis

59000 Lille

France

Telephone: + 33 3 20 49 44 13

Telefax: + 33 3 20 49 44 18

E-mail: chwedura@lfb.fr

Patient associations' contact points:

Leukaemia CARE

One Birch Court,
Blackpole East,
Worcester,
Worcestershire WR3 8SG
United Kingdom
Telephone: +44 1905 755977 / +44 845 521 3456
Telefax: +44 1905 755166
Email: info@leukaemiacare.org.uk

Ligue Nationale Contre le Cancer

14 Rue Corvisart
75013 Paris
France
Telephone: +33 1 53 55 24 00
Telefax: +33 1 43 36 91 10
E-mail: ligue@ligue-cancer.net

European Cancer Patient Coalition

ECPC Office
Am Rothenanger 1b
85521 Riemerling
Germany
Telephone: +49 89 628 36 807
Telefax: +49 89 628 36 808
E-mail: info@ecpc-online.org

**Translations of the active ingredient and indication in all official EU languages,
Norwegian and Icelandic**

Language	Active ingredient	Indication
English	Recombinant chimeric monoclonal antibody against CD20	Treatment of chronic lymphocytic leukaemia
Bulgarian	Рекомбинантно, химерно, моноклонално анти тяло срещу CD20.	Лечение на хронична лимфоцитна левкемия
Czech	Chimérická rekombinantní monoklonální protilátka CD20	Léčba chronické lymfatické leukémie
Danish	Rekombinant kimært monoklonalt anti-CD20 antistof	Behandling af kronisk lymfocytær leukæmi
Dutch	Recombinant chimerisch monoklonaal antilichaam gericht tegen CD20	Behandeling van chronische lymfocyttaire leukemie
Estonian	Kimeeriline rekombinantne monoklonaalne antikeha CD20 vastu	Kroonilise lümfoidleukeemia ravi
Finnish	Kimeerinen rekombinaattiteknikalla tehty monoklonaalinen vasta-aine	Kroonisen lymfosyyttileukemian hoito
French	Anticorps monoclonal chimérique recombinant anti-CD20	Traitement de la leucémie lymphoïde chronique
German	Chimärer, rekombinanter monoklonaler Antikörper gegen CD20	Behandlung der chronisch-lymphatischen Leukämie
Greek	Χιμαϊρικό ανασυνδυασμένο μονοκλωνικό αντίσωμα αντι- CD20	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας
Hungarian	Kimérás rekombináns monoklonális anti-CD20 antitest	Krónikus lymphoid leukémia kezelése
Italian	Anticorpo monoclonale ricombinante chimerico anti-CD20	Trattamento della leucemia linfocitica cronica
Latvian	Rekombinanta hīmēriskā monoklonāla pret-CD20 anti viela	Hroniskas limfocitiskās leikēmijas ārstēšana
Lithuanian	Rekombinantinis chimerinis monokloninis antikūnas prieš CD20	Lėtinės limfocitinės leukemijos gydymas
Maltese	Antikorp monoklonali kimeriku rikombinanti kontra CD20	Kura tal-lewkimja limfoċitika kronika
Polish	Chimeryczne, rekombinowane przeciwciało monoklonaalne przeciwko CD20	Leczenie przewlekłej białaczki limfatycznej
Portuguese	Anticorpo quimérico monoclonal recombinante anti-CD20	Tratamento da leucemia linfocítica crónica
Romanian	Anticorp monoclonal chimeric recombinant împotriva CD20	Tratamentul leucemiei limfoide cronice
Slovak	Rekombinantná chimérická monoklonálna protilátka proti CD20	Liečba chronickej lymfocytovej leukémie
Slovenian	Rekombinantno himerno monoklonsko protitelo CD20	Zdravljenje kronične limfatske levkemije
Spanish	Anticuerpo monoclonal recombinante quimérico anti-CD20	Tratamiento de la leucemia linfocítica crónica
Swedish	rekombinant chimär monoklonal antikropp riktad mot CD20	Behandling av kronisk lymfatisk leukemi
Norwegian	Rekombinant, kimærtmonoklonalt antistoff mot CD20	Behandling av kronisk lymfatisk leukemi
Icelandic	Kímerískt raðbrigða einstofna mótefni gegn CD20	Meðferð á langvinnu eítílfurmuhvítblæði