



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation lumiliximab for the treatment of chronic lymphocytic leukaemia

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in April 2010 on request of the sponsor.

On 4 February 2008, orphan designation EU/3/08/528 was granted by the European Commission to Biogen Idec Limited, United Kingdom, for lumiliximab for the treatment of chronic lymphocytic leukaemia.

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 cells called lymphocytes. 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 years. 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 is equivalent to a total of around 174,000 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 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.

Lumiliximab may be of potential significant benefit for the treatment of chronic lymphocytic leukaemia, because it might act differently from other medicinal products and may help kill cancer cells. 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?

Antibodies are proteins in the body that are able to recognise and bind to specific elements, the so called antigens. Antigens can be present on the surface of human cells (normal or cancer cells) or of microorganisms such as bacteria. Cancer cells typically have specific antigens in their surface; on the surface of the leukaemic cells, an antigen called CD23 is displayed. Lumiliximab is an antibody produced in a very specific way that results on antibodies with the same structure; these are called monoclonal antibodies. Lumiliximab has the ability to specifically recognise CD23 and therefore is able to bind to it. The union of lumiliximab and CD23 is ultimately thought to lead to a reaction in the cell that destroys the leukaemic cells bearing the CD23 molecule.

What is the stage of development of this medicine?

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

Lumiliximab 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 January 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 based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006).

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

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Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

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

¹ At the time of designation