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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Veltuzumab for the treatment of chronic lymphocytic leukaemia

On 29 January 2010, orphan designation (EU/3/09/713) was granted by the European Commission to Immunomedics GmbH, Germany, for veltuzumab 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, chronic lymphocytic leukaemia affected approximately 3.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 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

*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).

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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 veltuzumab might be of significant benefit for patients with CLL because results from studies in experimental models and early results from clinical studies indicate that it may 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.

How is this medicine expected to work?

Veltuzumab 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 to CD20, it is expected to cause the death of the cancerous B-lymphocytes.

What is the stage of development of this medicine?

The effects of veltuzumab 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, veltuzumab was not authorised anywhere in the EU for CLL. Orphan designation of veltuzumab had been granted in the United States of America for CLL.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 November 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

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

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