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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised single chain monoclonal antibody against CD37 for the treatment chronic lymphocytic leukaemia

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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 6 December 2012, orphan designation (EU/3/12/1083) was granted by the European Commission to Emergent Product Development UK Limited, United Kingdom, for humanised single chain monoclonal antibody against CD37 for the treatment 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 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 153,000 people, and is below the ceiling for orphan

*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 509,000,000 (Eurostat 2012).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

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 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 humanised single chain monoclonal antibody against CD37 might be of significant benefit for patients with CLL because it works in a different way to existing treatments and early studies show that it might improve the outcome 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?

This medicine 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. It has been designed to attach to CD37, which is present on the surface of B-lymphocytes. By attaching to CD37, the medicine is expected to cause the death of the cell. It is also expected to activate certain cells in the immune system (the body's natural defences), so that they kill the cancerous B-lymphocytes. This is expected to slow down the development of CLL.

What is the stage of development of this medicine?

The effects of humanised single chain monoclonal antibody against CD37 have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for CLL. Orphan designation of this medicine had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 November 2012 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:

Emergent Product Development UK Limited
Egham Police Station
High Street
Egham TW20 9HJ
United Kingdom
Telephone: +44 118 944 3300
Telefax: +44 118 944 3302
E-mail: info@ebsi.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 substance	Indication
English	Humanised single chain monoclonal antibody against CD37	Treatment of chronic lymphocytic leukaemia
Bulgarian	Хуманизирано моноклонално антитяло срещу CD37 (TRU-016) с единична верига	Лечение на хронична лимфоцитна левкемия
Czech	Humanizovaná monoklonální protilátka s jednoduchým řetězcem proti CD37 (TRU-016)	Léčba chronické lymfatické leukémie
Danish	Humaniseret enkeltkædet monoklonalt antistof rettet mod CD37 (TRU-016)	Behandling af kronisk lymfocytær leukæmi
Dutch	Gehumaniseerd enkel-keten monoklonaal antilichaam tegen CD37	Behandeling van chronische lymfocytaire leukemie
Estonian	CD37-vastane humaniseeritud üheahelaline monoklonaalne antikeha	Kroonilise lümfoidleukeemia ravi
Finnish	Humanisoitu yhden ketjun monoklonaalinen CD37:n vasta-aine (TRU-016)	Kroonisen lymfosyyttileukemian hoito
French	Anticorps monoclonal simple chaîne anti-CD37 humanisé (TRU-016)	Traitement de la leucémie lymphoïde chronique
German	Humanisierter einkettiger monoklonaler Antikörper gegen CD37	Behandlung der chronisch-lymphatischen Leukämie
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα μονής αλυσίδας έναντι του CD37	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας
Hungarian	CD37 (TRU-016) elleni humanizált, egyláncú, monoklonális antitest	Krónikus lymphoid leukémia kezelése
Italian	Anticorpo monoclonale umanizzato a singola catena anti-CD37 (TRU-016)	Trattamento della leucemia linfocitica cronica
Latvian	Humanizēta vienas ķēdes monoklonāla antiviela pret CD37	Hroniskas limfocitiskās leikēmijas ārstēšana
Lithuanian	Humanizuotas vienos grandinės monokloninis antikūnis prieš CD37 -	Lėtinės limfocitinės leukemijos gydymas
Maltese	Antikorp monoklonali umanizzat b'katina waħda kontra CD37	Kura tal-lewkimja limfoċitika kronika
Polish	Humanizowane jednołańcuchowe ludzkie przeciwciało monoklonalne przeciw CD37	Leczenie przewlekłej białaczki limfatycznej
Portuguese	Anticorpo monoclonal monocatenário humanizado anti-CD37 (TRU-016)	Tratamento da leucemia linfocítica crónica
Romanian	Anticorp monoclonal monocatenar umanizat anti-CD37 (TRU-016)	Tratamentul leucemiei limfoide cronice

[1] At the time of designation

Language	Active substance	Indication
Slovak	Humanizovaná monoklonálna protilátka proti CD37 s jednoduchým reťazcom	Liečba chronickej lymfocytovej leukémie
Slovenian	Humanizirano enoverižno monoklonalno protitelo proti CD37	Zdravljenje kronične limfatske levkemije
Spanish	Anticuerpo monoclonal de cadena única humanizado contra CD37	Tratamiento de la leucemia linfocítica crónica
Swedish	Humaniserad monoklonal enkelkedjeantikropp mot CD37	Behandling av kronisk lymfatisk leukemi
Norwegian	Humanisert enkeltkjedet monoklonalt antistoff mot CD37 (TRU-016)	Behandling av kronisk lymfatisk leukemi
Icelandic	Mannaaðlagað, einnar keðju, einstofna mótefni gegn CD37	Meðferð á langvinnu eitilfrumuhvítblæði