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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised Fc engineered monoclonal antibody against CD19 for the treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma

On 4 July 2014, orphan designation (EU/3/14/1286) was granted by the European Commission to MorphoSys AG, Germany, for humanised Fc engineered monoclonal antibody against CD19 for the treatment of chronic lymphocytic leukaemia/small lymphocytic lymphoma.

What is chronic lymphocytic leukaemia/small lymphocytic lymphoma?

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).

The disease known as 'small lymphocytic lymphoma' (SLL) is essentially the same disease as CLL. The name SLL is normally used when the cancer cells are located mainly in the lymph nodes.

CLL/SLL is the most common type of leukaemia and mainly affects older people. It is rare in people under the age of 40 years. CLL/SLL 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/SLL affected approximately 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 153,000 people[*], and is below the ceiling 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).

[*]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 28), Norway, Iceland and Liechtenstein. This represents a population of 511,100,000 (Eurostat 2014).

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What treatments are available?

Treatment for CLL/SLL 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/SLL is not causing any symptoms or is only getting worse very slowly may not need treatment. Treatment for CLL/SLL is only started if symptoms become troublesome. At the time of designation, the main treatment for CLL/SLL was chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with CLL/SLL because early studies show that it might improve the outcome of patients whose disease has come back after previous treatment. Laboratory studies have also shown a greater reduction in tumour growth when this medicine is used with other currently authorised medicines. 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 on certain cells in the body. It has been designed to attach to CD19, a protein that is present on the surface of the cancer cells. When attached to CD19, the medicine is expected to stimulate the body's natural defences to attack and kill the cancer cells and thereby slow down the progression of the disease.

What is the stage of development of this medicine?

The effects of the medicine 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/SLL were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for CLL/SLL or designated as an 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 14 May 2014 recommending the granting of this designation.

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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:

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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 ingredient	Indication
English	Humanised Fc engineered monoclonal antibody against CD19	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma
Bulgarian	Хуманизирано Fc разработено моноклонално антитяло срещу CD19	Лечение на хронична лимфоцитна левкемия/дребноклетъчен лимфоцитен лимфом
Croatian	Humanizirano Fc monoklonsko protutijelo protiv CD19	Liječenje kronične limfocitne leukemije/limfoma malih stanica
Czech	Humanizovaná upravená monoklonální protilátka Fc proti CD19	Léčba chronické lymfocytické leukémie / lymfomu z malých lymfocytů
Danish	Humaniseret Fc-fremstillet monoklonalt antistof mod CD19	Behandling af kronisk lymfocytær leukæmi/småcellet lymfocytært lymfom
Dutch	Gehumaniseerd Fc ontworpen monoklonaal antilichaam tegen CD19	Behandeling van chronische lymfocytaire leukemie/klein lymfocytair lymfoom
Estonian	CD19-vastane Fc-modifikatsiooniga humaniseeritud monoklonaalne antikeha	Kroonilise lümfotsütaarse leukeemia / väikserakulise lümfotsütaarse lümfoomi ravi
Finnish	Humanisoitu monoklonaalinen CD19-vasta-aine muunnellulla Fc-osalla	Kroonisen lymfosyyttisen leukemian/pienilymfosyyttisen lymfooman hoito
French	Anticorps monoclonal humanisé Fc anti-CD19 produit par génie génétique	Traitement de la leucémie lymphoïde chronique/du lymphome lymphocytaire à petites cellules
German	Humanisierter Fc-veränderter monoklonaler Antikörper gegen CD19	Behandlung der chronischen lymphatischen Leukämie / des kleinzelligen lymphozytischen Lymphoms
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα έναντι του CD19, με τροποιημένο τμήμα Fc	Θεραπεία χρόνιας λεμφοκυτταρικής λευχαιμίας/ λεμφώματος από μικρά λεμφοκύτταρα
Hungarian	Humanizált, Fc-optimalizált, CD19 elleni monoklonális antitest	Krónikus lymphocytás leukaemia/kis lymphocytás lymphoma kezelése
Italian	Anticorpo monoclonale umanizzato ingegnerizzato Fc, diretto contro CD19	Trattamento della leucemia linfatica cronica/ linfoma a piccoli linfociti
Latvian	Humanizēta Fc inženierēta monoklonāla antiviela pret CD19	Hroniskas limfocitārās leikēmijas/mazo šūnu limfocitārās limfomas ārstēšana
Lithuanian	Sukonstruotas humanizuotas Fc monokloninis antikūnas prieš CD19	Lėtinės limfocitinės leukemijos / smulkių limfocitų limfomos gydymas
Maltese	Antikorp monoklonali Fc umanizzat maħdum kontra CD19	Kura tal-lewkimja limfoċitika kronika/limfoma limfoċitika żgħira
Polish	Humanizowane przeciwciało monoklonalne anty-CD19 ze zmodyfikowaną domeną Fc	Leczenie przewlekłej białaczki limfatycznej/chłoniaków limfocytarnych z małych limfocytów

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	Anticorpo monoclonal Fc humanizado anti CD19	Tratamento da leucemia linfocítica crónica/ linfoma linfocítico de pequenas células
Romanian	Anticorp monoclonal umanizat Fc anti-CD19 produs prin inginerie genetică	Tratamentul leucemiei limfocitare cronice/limfomului limfocitar cu celule mici
Slovak	Humanizovaná Fc upravená monoklonálna protilátka proti CD19	Liečba chronickej lymfocytovej leukémie / lymfómu z malých lymfocytov
Slovenian	Humanizirano monoklonalno protitelo proti receptorjem CD19 Fc	Zdravljenje kronične limfocitne levkemije/drobnoceličnega limfocitnega limfoma
Spanish	Anticuerpo monoclonal Fc humanizado contra CD19	Tratamiento de la leucemia linfocítica crónica/del linfoma linfocítico pequeño
Swedish	Humaniserad Fc-framställd monoklonal antikropp riktad mot CD19	Behandling av kronisk lymfocytisk leukemi /småcelligt lymfocytiskt lymfom
Norwegian	Humanisert Fc konstruert monoklonalt antistoff mot CD19	Behandling av kronisk lymfatisk leukemi / småcellet lymfocytært lymfom
Icelandic	Mannaaðlagað Fc-útbúið einstofna mótefni gegn CD19	Meðferð við langvinnu eitilfrumuhvítblæði/smáeitilfrumukrabbameini