



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

2 March 2015
EMA/COMP/793170/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised Fc engineered monoclonal antibody against CD19 for the treatment of diffuse large B-cell lymphoma

On 15 January 2015, orphan designation (EU/3/14/1424) was granted by the European Commission to MorphoSys AG, Germany, for humanised Fc engineered monoclonal antibody against CD19 for the treatment of diffuse large B-cell lymphoma.

What is diffuse large B-cell lymphoma?

Diffuse large B-cell lymphoma is the most common cancer of the lymphatic system, a network of vessels that transport fluid (lymph) from tissues through the lymph nodes and into the bloodstream. The cancer affects a type of white blood cell called B lymphocytes, or B cells. In patients with this cancer, the B cells multiply too quickly and live for too long, so there are too many of them in the lymph nodes. The first sign of the disease is usually a lump in the neck, under the arm or in the groin area, which is caused by an enlarged lymph node. Patients with diffuse large B-cell lymphoma may also have fever, tiredness, night sweats or weight loss that have no obvious cause.

Although some people with diffuse large B-cell lymphoma can be cured, it remains a serious and life-threatening disease, particularly when the disease is diagnosed late or has come back after initial treatment.

What is the estimated number of patients affected by the condition?

At the time of designation, diffuse large B-cell lymphoma affected approximately 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 128,000 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).



What treatments are available?

At the time of designation, several medicines were authorised for the treatment of diffuse large B-cell lymphoma in the EU. The main treatment was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation). Autologous haematopoietic (blood) stem cell transplantation was also used in patients at risk of the disease coming back after treatment. This is a complex procedure where patients receive their own stem cells to help restore the bone marrow.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with diffuse large B-cell lymphoma because early studies show a positive response in patients whose disease has come back after, or who have not responded to, previous treatment. 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 diffuse large B-cell lymphoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for diffuse large B-cell lymphoma 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 11 December 2014 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Humanised Fc engineered monoclonal antibody against CD19	Treatment of diffuse large B-cell lymphoma
Bulgarian	Хуманизирано Fc разработено моноклонално антитяло срещу CD19	Лечение на дифузен В-едроклетъчен лимфом
Croatian	Humanizirano Fc monoklonsko protutijelo protiv CD19	Liječenje difuznog limfoma velikih B-stanica
Czech	Humanizovaná upravená monoklonální protilátka Fc proti CD19	Léčba velkobuněčného difuzního B-lymfomu
Danish	Humaniseret Fc-fremstillet monoklonalt antistof mod CD19	Behandling af diffust storcellet B-celle lymfom
Dutch	Gehumaniseerd Fc ontworpen monoklonaal antilichaam tegen CD19	Behandeling van diffuus grootcellig B-cel-lymfoom
Estonian	CD19-vastane Fc-modifikatsiooniga humaniseeritud monoklonaalne antikeha	Diffuusse suure β-rakulise lümfoomi ravi
Finnish	Humanisoitu monoklonaalinen CD19-vasta-aine muunnellulla Fc-osalla	Diffuusin suurisoluisen B-solulymfooman hoito
French	Anticorps monoclonal humanisé Fc anti-CD19 produit par génie génétique	Traitement du lymphome diffus à grandes cellules B
German	Humanisierter Fc-veränderter monoklonaler Antikörper gegen CD19	Behandlung des diffusen großzelligen B-Zell-Lymphoms
Greek	Ανθρώποποιημένο μονοκλωνικό αντίσωμα έναντι του CD19, με τροποποιημένο τμήμα Fc	Θεραπεία του διάχυτου μεγαλοκυτταρικού λεμφώματος Β-κυττάρου (DLBCL)
Hungarian	Humanizált, Fc-optimalizált, CD19 elleni monoklonális antitest	Diffúz nagy B-sejtes lymphoma kezelése
Italian	Anticorpo monoclonale umanizzato ingegnerizzato Fc, diretto contro CD19	Terapia del Linfoma non-Hodgkin diffuso a grandi cellule di tipo B (DLBCL)
Latvian	Humanizēta Fc inženierēta monoklonāla antivielā pret CD19	Difūzas lielo B šūnu limfomas ārstēšana
Lithuanian	Sukonstruotas humanizuotas Fc monokloninis antikūnas prieš CD19	Difuzinės stambiųjų B ląstelių limfomos gydymas
Maltese	Antikorp monoklonali Fc umanizzat maħdum kontra CD19	Kura tal-linfoma tač-ċelluli tat-tip B kbar mxerrda
Polish	Humanizowane przeciwciało monoklonalne anty-CD19 ze zmodyfikowaną domeną Fc	Leczenie rozlanego chłoniaka z dużych limfocytów B
Portuguese	Anticorpo monoclonal Fc humanizado anti CD19	Tratamento do linfoma difuso de grandes células B
Romanian	Anticorp monoclonal umanizat Fc anti-CD19 produs prin inginerie genetică	Tratamentul limfomului difuz cu celule B mari

¹ At the time of designation

Language	Active ingredient	Indication
Slovak	Humanizovaná Fc upravená monoklonálna protilátka proti CD19	Liečba difúzneho veľkobunkového lymfómu z buniek B
Slovenian	Humanizirano monoklonalno protitelo proti receptorjem CD19 Fc	Zdravljenje razširjenega limfoma velikih B celic
Spanish	Anticuerpo monoclonal Fc humanizado contra CD19	Tratamiento del linfoma difuso de células B grandes
Swedish	Humaniserad Fc-framställd monoklonal antikropp riktad mot CD19	Behandling av diffusa storcelliga B-cells lymfom
Norwegian	Humanisert Fc konstruert monoklonalt antistoff mot CD19	Behandling av diffust storcellet B-celle lymfom
Icelandic	Mannaaðlagað Fc-útbúið einstofna mótefni gegn CD19	Til meðferðar á dreifðu stórfrumu B frumu eitlakrabbameini