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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous human peripheral blood Vdelta1+ T lymphocytes activated in vitro by cytokine and monoclonal antibody treatment for the treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma

On 9 October 2015, orphan designation (EU/3/15/1566) was granted by the European Commission to Lymphact - Lymphocyte Activation Technologies S.A., Portugal, for autologous human peripheral blood Vdelta1+ T lymphocytes activated in vitro by cytokine and monoclonal antibody treatment 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 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 the same disease as CLL. The name SLL is 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 4.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 246,000 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 512,900,000 (Eurostat 2015).

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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 getting worse only very slowly may not need treatment. Treatment for CLL/SLL is started only 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 in experimental models show that it might be effective at killing the cancer cells. 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 contains certain T cells taken from the patient. T cells are cells of the immune system (the body's natural defences) that kill infected or abnormal cells. To make this medicine, the T cells taken from the patient are purified to obtain certain T cells called Vdelta1+ T cells. When the medicine is injected back into the patient, Vdelta1+ T cells are expected to attach to the cancerous B lymphocytes in CLL/SLL and kill them.

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, no clinical trials with the medicine in patients with CLL/SLL had been started.

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 3 September 2015 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

For details of the current sponsor of the orphan designation please refer to the information on the main web page of this Public Summary of Opinion.

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	Autologous human peripheral blood Vdelta1+ T lymphocytes activated in vitro by cytokine and monoclonal antibody treatment	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma
Bulgarian	Автоложни човешки периферни кръвни Vδ1+ Т-лимфоцити, активирани ин витро чрез третиране с цитокин и моноклонално антитяло	Лечение на хронична лимфоцитна левкемия / Дребноклетъчен лимфоцитен лимфом
Croatian	Autologni ljudski Vdelta1+ T limfociti iz periferne krvi, aktivirani in vitro obradom citokinima i monoklonskim protutijelima	Liječenje kronične limfocitne leukemije / limfoma malih limfocita
Czech	Autologní Vdelta1+ T lymfocyty humanní periferní krve aktivovány in vitro cytokiny a monoklonálními protilátkami.	Léčba chronické lymfatické leukémie/lymfom z malých lymfocytů
Danish	Autologe humane perifert blod Vdelta1+ T-lymfocytter aktiveret in vitro ved behandling med cytokin og monoklonalt antistof	Behandling af kronisk lymfatisk leukæmi/ Småcellet lymfocytisk lymfom
Dutch	Autologe menselijk perifeer bloed Vdelta1+ T-lymfocyten in vitro geactiveerd door behandeling met cytokine en monoklonaal antilichaam	Behandeling van chronische lymfocytaire leukemie/ kleincellig lymfocytair lymfoom
Estonian	Autoloogsed inimese perifeerse vere Vdelta1+ T lümfotsüüdid, mida on aktiveeritud in vitro tsütokiinide ja monoklonaalse antikeha raviga	Kroonilise lümfoidleukeemia ja väikerakk-lümfotsüütlümfoomi ravi
Finnish	Autologiset ihmisen perifeerisestä verestä peräisin olevat in vitro sytokiinillä ja monoklonaalisella vasta-aine hoidolla aktivoidut Vdelta1+ T-lymfosyytit	Kroonisen lymfaattisen leukemian japienilymfosyyttisen lymfooman hoito
French	Lymphocytes Vdelta1+ T autologues du sang humain périphérique activées in vitro par traitement avec cytokines et anticorps monoclonaux	Traitement de la leucémie lymphoïde chronique/du lymphome lymphocytaire à petites cellules
German	Autologe menschliche periphere Blut Vdelta1+ T-Lymphozyten, in vitro durch Cytokin- und monoklonaler Antikörper-Behandlung aktiviert	Behandlung der chronischen lymphatischen Leukämie/des kleinzelligen lymphozytischen Lymphoms

[1] At the time of designation

Language	Active ingredient	Indication
Greek	Autologous human peripheral blood Vdelta1+ T lymphocytes activated in vitro by cytokine and monoclonal antibody treatment Αυτόλογα ανθρώπινα Vδ1+ Τ λεμφοκύτταρα περιφερικού αίματος, ενεργοποιημένα in vitro με επώαση με κυτταροκίνες και μονοκλωνικά ατνισώματα.	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας/ του Λεμφώματος από Μικρά Λεμφοκύτταρα
Hungarian	Autológ humán perifériás vér Vdelta1+ cytokinnel in vitro aktivált monoklonális antitest-kezelés	Krónikus lymphoid leukaemia/kissejtes lymphocytás lymphoma kezelése
Italian	Linfociti Vdelta1+ T autologhi di sangue umano periferico attivati in vitro mediante trattamento con citochina e anticorpo monoclonale	Trattamento della leucemia linfocitica cronica/ linfoma a piccoli linfociti
Latvian	Autologi cilvēka perifēro asiņu Vdelta1+ T limfocīti, kas aktivizēti *in vitro*, tos apstrādājot ar citokīniem un monoklonālām antivielām	Hroniskas limfoleikozes/mazo limfocītu limfomas ārstēšana
Lithuanian	Autologiniai žmogaus periferinio kraujo Vdelta1+ T limfocitai, aktyvuoti *in vitro* veikiantcitokinu ir monokloniniais antikūnais	Lėtinės limfocitinės leukemijos/ smulkių limfocitų limfomos gydymas
Maltese	Limfoċiti tat-tip Vdelta1+ T awtologużi minn demm periferali uman attivati in vitro permezz ta' trattament biċ-ċitokini u antikorpi monoklonali	Kura tal-lewkimja limfoċitika kronika/limfoma limfoċitika żgħira
Polish	Autologiczne limfocyty ludzkiej krwi obwodowej Vdelta1+ T aktywowane in vitro przez działanie cytokiny i przeciwciała monoklonalnego	Leczenie przewlekłej białaczki limfatycznej/chłoniaka z małych limfocytów
Portuguese	Linfócitos Vdelta1+ T autólogos de sangue humano periférico ativados *in vitro* por tratamento com citocina e anticorpo monoclonal	Tratamento da leucemia linfocítica crónica/linfoma de pequenos linfócitos
Romanian	Limfocite T Vdelta1+ autologe din sângele uman periferic, activate in vitro prin tratament cu citokine și anticorpi monoclonali	Tratamentul leucemiei limfoide cronice / limfomului limfocitar cu celule mici
Slovak	Autológne ľudské periférnekrvné Vdelta1+ T lymfocyty aktivované in vitro pôsobením cytokínov a monoklonálnych protilátok	Liečba chronickej lymfocytovej leukémie / malobunkového lymfocytového lymfómu
Slovenian	Avtologni človeški limfociti Vdelta1+ T, aktivirani in vitro s citokini in monoklonskimi protitelesi	Zdravljenje kronične limfatične levkemije / drobnoceličnega limfocitnega limfoma
Spanish	Linfocitos Vdelta1+ T autologos de la sangre humana periferica activados *in vitro* por tratamiento com citocinas y anticuerpo monoclonal	Tratamiento de la leucemia linfocítica crónica linfoma linfocítico pequeño

Language	Active ingredient	Indication
Swedish	Autolog Vdelta1+ T-lymfocyter från humant perifert blod aktiverade in vitro genom behandling med cytokin och monoklonal antikropp	Behandling av kronisk lymfatisk leukemi/småcelligt lymfocytärt lymfom
Norwegian	Autologe Vdelta1+ T-lymfocytter fra humant perifert blod aktivert in vitro ved behandling med cytokin og monoklonalt antistoff	Behandling av kronisk lymfatisk leukemi / småcellet lymfocytært lymfom
Icelandic	Samgena Vdelta1+ T eitilfrumur úr manna útæðablóði virkjað in vitro með meðhöndlun með sýtókín og einstofna mótefni	Meðferð á langvinnu eitilfrumuhvítblæði/ smáeitilfrumu eitlakrabbameini