



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Inecalcitol for the treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma

On 16 January 2014, orphan designation (EU/3/13/1223) was granted by the European Commission to Hybrigenics SA, France, for inecalcitol 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 cells 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, where blood cells are produced). 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 mainly affects older people and is rare in people under the age of 40 years. It is a long-term debilitating and life-threatening disease because it involves the weakening of the immune system (the body's natural defences), which can leave patients vulnerable to severe infections.

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

At the time of designation, CLL/SLL affected approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 179,000 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 512,200,000 (Eurostat 2013).



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 clinical studies show that it might improve the outcome for patients and that it could delay the progression of the disease. 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?

Inecalcitol is a type of vitamin D (a 'vitamin D analogue') and attaches to the vitamin D receptor which is involved in turning genes 'on' and 'off' within cells. In CLL/SLL, inecalcitol is expected to switch 'on' the genes that suppress the division and growth of the tumour cells and to switch 'off' those genes that stimulate the division and growth of the tumour cells. This is expected to lead to a reduction in the growth and division of the cancer cells, thereby slowing the progression of the disease.

What is the stage of development of this medicine?

The effects of inecalcitol have been evaluated in experimental models.

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

At the time of submission, inecalcitol 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 12 December 2013 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	Inecalcitol	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma
Bulgarian	инекалцитол	Лечение на хронична лимфоцитна левкемия/ малък лимфоцитен лимфом
Czech	Inecalcitol	Léčba chronické lymfatické leukémie/lymfom z malých lymfocytů
Croatian	Inekalcitol	Liječenje kronične limfocitne leukemije/ limfoma malih stanica
Danish	Inecalcitol	Behandling af kronisk lymfatisk leukæmi/småcellet lymfocytært lymfom
Dutch	Inecalcitol	Behandeling van chronische lymfatische leukemie/kleincellig lymfocytair lymfoom
Estonian	Inecalcitol	Krooniline lümfoidne leukeemia/ väike-lümfotsüütümfoomi ravi
Finnish	Inekalsitoli	Kroonisen lymfosyyttileukemian ja pienisoluisen lymfosyyttisen lymfooman hoito
French	Inecalcitol	Traitement de la leucémie lymphoïde chronique/du lymphome lymphocytaire à petites cellules
German	Inecalcitol	Behandlung der chronischen lymphatischen Leukämie/des kleinzelligen lymphozytischen Lymphoms
Greek	Ινεκαλσιτόλη	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας/ λεμφώματος από μικρά λεμφοκύτταρα
Hungarian	Inekalcitol	Krónikus lymphoid leukaemia kezelése/ kissejtes lymphocytás lymphoma kezelése
Italian	Inecalcitol	Trattamento della leucemia linfocitica cronica/linfoma linfocitico a piccole cellule
Latvian	Inekalcitols	Hroniskas limfoleikozes/mazo limfocītu limfomas ārstēšana
Lithuanian	Inekalcitolis	Lėtinės limfocitinės leukemijos/ smulkių limfocitų limfomos gydymas
Maltese	Inecalcitol	Trattament tal-lewkimja limfoċitika kronika/ limfoma limfoċitika żgħira
Polish	Inekalcytol	Leczenie przewlekłej białaczki limfocytowej/ chłoniaka z małych limfocytów
Portuguese	Inecalcitol	Tratamento da leucemia linfocítica crónica/linfoma linfocítico de células B pequenas
Romanian	Inecalcitol	Tratamentul leucemiei limfocitare cronice/limfomului limfocitar cu celule mici
Slovak	Inekalcitol	Liečba chronickej lymfatickej leukémie/lymfómu z malých lymfocytov
Slovenian	Inekalcitol	Zdravljenje kronične limfatične levkemije

¹ At the time of designation

Language	Active ingredient	Indication
Spanish	Inecalcitol	Tratamiento de la leucemia linfocítica crónica/ linfoma con pequeños linfocíticos
Swedish	Inecalcitol	Behandling av kronisk lymfatisk leukemi /småcelligt lymfocytärt lymfom
Norwegian	Inekalsitol	Behandling av kronisk lymfatisk leukemi/småcellet lymfocytært lymfom
Icelandic	Ínekalsitól	Meðferð á langvinnu eitilfrumuhvítblæði/smáfrumu eitilfrumueitilfrumukrabbameini