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EMA/COMP/637216/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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

On 19 November 2014, orphan designation (EU/3/14/1354) was granted by the European Commission to Clinipace GmbH, Germany, for selinexor 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 less than 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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 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 who have undergone extensive treatment before, including a subset of CLL patients with bad prognosis (those with the so-called 'Richter syndrome'). 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 expected to work by blocking the action of a protein called exportin 1 (XPO1). XPO1 is found at high levels in many cancer cells, where it prevents the actions of proteins that help stop cancer growth. By blocking XPO1, the medicine is expected to enhance the action of these anti-cancer proteins and bring about the death of the cancer cells, thereby slowing 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 CCL/SLL were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for CCL/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 9 October 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	Selinexor	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma
Bulgarian	Селинексор	Лечение на хронична лимфоцитна левкемия / Дребноклетъчен лимфоцитен лимфом
Croatian	Selineksor	Liječenje kronične limfocitne leukemije / limfoma malih limfocita
Czech	Selinexor	Léčba chronické lymfatické leukémie/lymfom z malých lymfocytů
Danish	Selinexor	Behandling af kronisk lymfatisk leukæmi/ Småcellet lymfocytisk lymfom
Dutch	Selinexor	Behandeling van chronische lymfocytaire leukemie/ kleincellig lymfocytair lymfoom
Estonian	Selinexor	Kroonilise lümfoidleukeemia ja väikerakk-lümfotsüütlümfoomi ravi
Finnish	Selinexor	Kroonisen lymfaattisen leukemian japienilymfosyyttisen lymfooman hoito
French	Sélinexor	Traitement de la leucémie lymphoïde chronique/du lymphome lymphocytaire à petites cellules
German	Selinexor	Behandlung der chronischen lymphatischen Leukämie/des kleinzelligen lymphozytischen Lymphoms
Greek	Σελινεξόρη	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας/ του Λεμφώματος από Μικρά Λεμφοκύτταρα
Hungarian	Szelinexor	Krónikus lymphoid –leukaemia/kissejtes lymphocytás lymphoma kezelése
Italian	Selinexor	Trattamento della leucemia linfocitica cronica/ linfoma a piccoli linfociti
Latvian	Selinexor	Hroniskas limfoleikozes/mazo limfocītu limfomas ārstēšana
Lithuanian	Selineksoras	Lėtinės limfocitinės leukemijos/ smulkių limfocitų limfomos gydymas
Maltese	Selinexor	Kura tal-lewkimja limfoċitika kronika/limfoma limfoċitika żgħira
Polish	Selineksor	Leczenie przewlekłej białaczki limfatycznej/chłoniaka z małych limfocytów
Portuguese	Selinexor	Tratamento da leucemia linfocítica crónica/linfoma de pequenos linfócitos
Romanian	Selinexor	Tratamentul leucemiei limfoide cronice / limfomului limfocitar cu celule mici
Slovak	Selinexor	Liečba chronickej lymfocytovej leukémie / malobunkového lymfocytového lymfómu
Slovenian	Selineksor	Zdravljenje kronične limfatične levkemije / drobnoceličnega limfocitnega limfoma
Spanish	Selinexor	Tratamiento de la leucemia linfocítica crónica linfoma linfocítico pequeño

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Selinexor	Behandling av kronisk lymfatisk leukemi/småcelligt lymfocytärt lymfom
Norwegian	Selineksor	Behandling av kronisk lymfatisk leukemi / småcellet lymfocytært lymfom
Icelandic	Selinexor	Meðferð á langvinnu eitilfrumuhvítblæði/ smáeitilfrumu eitlakrabbameini