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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cenersen for the treatment chronic lymphocytic leukaemia

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 3 December 2008, orphan designation (EU/3/08/587) was granted by the European Commission to EleosInc Limited, United Kingdom, for cenersen for the treatment chronic lymphocytic leukaemia.

What is chronic lymphocytic leukaemia?

Chronic lymphocytic leukaemia is a disease in which cancer cells are found in the blood and the bone marrow. The bone marrow is the spongy tissue inside the large bones in the body. Normally, the bone marrow makes cells, called "blasts", which mature into several different types of blood cells with specific functions in the body. These include red cells, white cells and platelets. Red blood cells carry oxygen and other materials to all tissues of the body. White blood cells fight infection. Platelets support blood clotting. When leukaemia develops, the bone marrow produces large numbers of abnormal blood cells. Over a period of time these abnormal cells replace the normal white cells, red cells and platelets in the bone marrow, which reduces the number of normal cells in the blood and leads to anaemia, coagulation problems (bruising, haemorrhages) and repeated infections. There are several types of leukaemias. Chronic lymphocytic leukaemia is a cancer of a type of white blood cells called B-lymphocytes. The lymphocytes multiply and live too long, so there are too many of them circulating in the blood. These leukaemic lymphocytes look normal, but they are not fully developed and do not work properly. Chronic lymphocytic leukaemia is the most common type of leukaemia; it mainly affects older people, being rare in people under the age of 40. Chronic lymphocytic leukaemia is chronically debilitating and life-threatening, due to the severe prognosis and the poor long-term survival for high-risk patients.

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

At the time of designation, chronic lymphocytic leukaemia affected between 1 and 3 in 10,000 people in the European Union (EU). This was equivalent to a total of between 50,000 and 151,000 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).

What treatments are available?

Treatment for leukaemia is complex, and depends on a number of factors including the type of leukaemia, the extent of the disease and whether the leukaemia has been treated before. It also depends on the age, symptoms, and the general health of the patient. Some people with B-cell chronic lymphocytic leukaemia never require treatment, if their illness is not causing any symptoms and is progressing slowly. Treatment is often started only if and when the symptoms become troublesome. Current main treatment of chronic lymphocytic leukaemia is chemotherapy (using drugs to kill cancer cells). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation.

How is this medicine expected to work?

The cells have natural mechanisms that induce their death when some alterations occur, for instance when their genetic material is damaged. One of the mechanisms of cell death is controlled by a gene called *p53*. This gene makes cells to undergo either repair of the damages or cell death. In cancer cells, due to a complicated association of alterations, *p53* activation tends to result in repair even though this is not complete and finally leads to production of more damaged cells and therefore multiplication of the tumour. The product is able to block both *p53* and mutated *p53*; by doing this it blocks *p53* mediated cell repair cancer so cells can undergo cell death via an alternative pathway.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with chronic lymphocytic leukaemia were ongoing.

At the time of submission, cenersen was not authorised anywhere in the world for chronic lymphocytic leukaemia.

Orphan designation of cenersen had been granted in the United States for treatment of chronic lymphocytic leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 October 2008 recommending the granting of this designation.

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

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

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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	Cenersen	Treatment of chronic lymphocytic leukaemia
Bulgarian	Ценерзен	Лечение на хронична лимфоцитна левкемия
Czech	Cenersen	Léčba chronické lymfatické leukémie
Danish	Cenersen	Behandling af kronisk lymfocytær leukæmi
Dutch	Cenersen	Behandeling van chronische lymfocytaire leukemie
Estonian	Cenersen	Kroonilise lümfoidleukeemia ravi
Finnish	Cenersen	Kroonisen lymfosyyttileukemian hoito
French	Cénersen	Traitement de la leucémie lymphoïde chronique
German	Cenersen	Behandlung der chronisch-lymphatischen Leukämie
Greek	Σενερσέν	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας
Hungarian	Cenersen	Krónikus lymphoid leukémia kezelése
Italian	Cenersen	Trattamento della leucemia linfocitica cronica
Latvian	Cenerzēns	Hroniskas limfocitiskās leikēmijas ārstēšana
Lithuanian	Cenersenas	Lėtinės limfocitinės leukemijos gydymas
Maltese	Cenersen	Kura tal-lewkimja limfoċitika kronika
Polish	Cenersen	Leczenie przewlekłej białaczki limfatycznej
Portuguese	Cenersen	Tratamento da leucémia linfocítica crónica
Romanian	Cenersen	Tratamentul leucemiei limfoide cronice
Slovak	Cenersén	Liečba chronickej lymfocytovej leukémie
Slovenian	Cenersen	Zdravljenje kronične limfatske levkemije
Spanish	Cenersén	Tratamiento de la leucemia linfocítica crónica
Swedish	Cenersen	Behandling av kronisk lymfatisk leukemi
Norwegian	Cenersen	Behandling av kronisk lymfatisk leukemi
Icelandic	Cenersen	Meðferð á langvinnu eitilfrumuhvítblæði

[1] At the time of designation