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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lenalidomide for the treatment of chronic lymphocytic leukaemia

On 19 November 2007, orphan designation (EU/3/07/494) was granted by the European Commission to Celgene Europe Limited, UK, for lenalidomide for the treatment of 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.

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

At the time of designation, chronic lymphocytic leukaemia affected approximately 3.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 174,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and 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 27), Norway, Iceland and Liechtenstein. This represents a population of 498,000,000 (Eurostat 2006).

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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 have 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.

Lenalidomide could be of potential significant benefit for the treatment of chronic lymphocytic leukaemia, because it might act differently from other medicinal products. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Cancers need to produce a network of new blood vessels in order to grow. Without forming these blood vessels, cancers cannot grow. The theory is that lenalidomide will prevent the tumour from growing, by preventing the development of new blood vessels and possibly also reducing the supply of oxygen and nutrients to the cancer cells. Lenalidomide is probably also an immunomodulator; in other words it acts by modifying the activity of the cells from the defence system involved in fighting infections and "foreign" cells / tissues (such as bacteria and tumours). This might result in an effect on the tumour growth and survival of the tumour cells.

What is the stage of development of this medicine?

The effects of lenalidomide were evaluated in experimental models.

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

Lenalidomide was not authorised anywhere in the world for chronic lymphocytic leukaemia or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 September 2007 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 languages[1], Norwegian and Icelandic

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

[1] At the time of designation