



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Dinaciclib for the treatment of chronic lymphocytic leukaemia

On 27 September 2011, orphan designation (EU/3/11/901) was granted by the European Commission to Merck Sharp & Dohme Limited, United Kingdom, for dinaciclib for the treatment of chronic lymphocytic leukaemia.

What is chronic lymphocytic leukaemia?

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 blood cells, red blood cells and platelets (components that help the blood to clot) in the bone marrow (the spongy tissue inside the large bones in the body).

CLL is the most common type of leukaemia and mainly affects older people. It is rare in people under the age of 40 years. CLL 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, chronic lymphocytic leukaemia affected approximately 3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 152,000 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 CLL 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 is not causing any symptoms or is only getting worse very slowly may not need

*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 506,300,000 (Eurostat 2011).



treatment. Treatment for CLL is only started if symptoms become troublesome. At the time of designation, the main treatment for CLL was chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that dinaciclib might be of significant benefit for patients with chronic lymphocytic leukaemia because it works in a different way to existing treatment and early studies indicate that it may improve the outcome of patients with this condition. 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?

Dinaciclib blocks the action of specific enzymes (specialised proteins) called cyclin-dependent kinases (CDK), which are involved in regulating the cell cycle. The cell cycle is the series of events that takes place in a cell leading to its division and replication in the body. In order to become active, CDK has to attach to proteins called cyclins. In cancer cells, there are many of these cyclins and CDK becomes abnormally active leading to the growth and spread of cancer cells. By targeting CDK and blocking its action, dinaciclib is expected to interfere with the cell cycle of cancer cells in CLL and to bring about their death.

What is the stage of development of this medicine?

The effects of dinaciclib have been evaluated in experimental models.

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

At the time of submission, dinaciclib was not authorised anywhere in the EU for CLL 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 15 July 2011 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	Dinaciclib	Treatment of chronic lymphocytic leukaemia
Bulgarian	Динациклиб	Лечение на хронична лимфоцитна левкемия
Czech	Dinaciclib	Léčba chronické lymfatické leukémie
Danish	Dinaciclib	Behandling af kronisk lymfocytær leukæmi
Dutch	Dinaciclib	Behandeling van chronische lymfocyttaire leukemie
Estonian	Dinatsikliib	Kroonilise lümfoidleukeemia ravi
Finnish	Dinasiklibi	Kroonisen lymfaattisen leukemian hoito
French	Dinaciclib	Traitement de la leucémie lymphoïde chronique
German	Dinaciclib	Behandlung der chronisch-lymphatischen Leukämie
Greek	Δινασικλίβη	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας
Hungarian	Dinaciklib	Krónikus lymphoid leukémia kezelése
Italian	Dinaciclib	Trattamento della leucemia linfocitica cronica
Latvian	Dinaciklibs	Hroniskas limfocītiskās leukēmijas ārstēšana
Lithuanian	Dinaciklibas	Lėtinės limfocitinės leukemijos gydymas
Maltese	Dinaciclib	Kura tal-lewkimja limfoċitika kronika
Polish	Dynacyklib	Leczenie przewlekłej białaczki limfatycznej
Portuguese	Dinaciclib	Tratamento da leucemia linfocítica crónica
Romanian	Dinaciclib	Tratamentul leucemiei limfoide cronice
Slovak	Dinaciklib	Liečba chronickej lymfocytovej leukémie
Slovenian	Dinaciklib	Zdravljenje kronične limfatske levkemije
Spanish	Dinaciclib	Tratamiento de la leucemia linfocítica crónica
Swedish	Dinaciklib	Behandling av kronisk lymfatisk leukemi
Norwegian	Dinasiklib	Behandling av kronisk lymfatisk leukemi
Icelandic	Dínacíclíb	Meðferð á langvinnu eitilfrumuhvítblæði

¹ At the time of designation