



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') for the treatment of 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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2013 on request of the Sponsor.

On 20 November 2001, orphan designation EU/3/01/063 was granted by the European Commission to Voisin Consulting S.A.R.L., France, for deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') for the treatment of chronic lymphocytic leukemia.

The sponsorship was transferred to Avensis Pharma in January 2003 and back to Voisin Consulting S.A.R.L. in July 2005.

What is chronic lymphocytic leukemia?

Chronic lymphocytic leukemia 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" that mature into several different types of blood cells that have 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 make the blood clot. When leukaemia develops, the bone marrow produces large numbers of abnormal blood cells. There are several types of leukaemias. Chronic lymphocytic leukemia is a cancer of the white blood cells called lymphocytes. The lymphocytes multiply too quickly and live too long, so there are too



many of them circulating in the blood. These leukemic lymphocytes look normal, but they are not fully developed and do not work properly. Over a period of time these abnormal cells replace the normal white cells, red cells and platelets in the bone marrow. It is the most common type of leukemia. Chronic lymphocytic leukemia mainly affects older people and is rare in people under the age of 40. Chronic lymphocytic leukemia 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 leukemia affected approximately 2.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 91,000 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 leukemia is complex and depends on a number of factors including the type of leukemia, the extent of the disease and whether the leukemia has been treated before. It also depends on the age, the symptoms, and the general health of the patient. Some people with chronic lymphocytic leukemia never have treatment if their illness is not causing any symptoms and is progressing only very slowly. Treatment is only started if and when the symptoms become troublesome. Current main treatment of chronic lymphocytic leukemia 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.

Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') could be of potential significant benefit for the treatment of chronic lymphocytic leukemia. The potential significant benefit is mainly because it might improve the long-term outcome of the patients. 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?

The so-called programmed cell death (apoptosis) is a process that is very important for the natural death of cells. A certain protein (B-cell lymphoma-associated protein 2) participates in regulating this process of cell death. In chronic lymphocytic leukemia there is too much of this protein and the cells live longer than normal. Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') might prevent the formation of this protein, decrease the amount of protein and might thereby restore the normal cell death.

What is the stage of development of this medicine?

The effects of deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') were evaluated in experimental models.

At the time of submission of the application for orphan designation, several clinical trials in patients with chronic lymphocytic leukemia were completed and some were ongoing.

Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') was not marketed anywhere worldwide for chronic lymphocytic leukemia, at the time of submission.

*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.
At the time of designation, this represented a population of 378,800,000 (Eurostat 2001).

Orphan designation of deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') was pending in the United States of America.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 2001 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	Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3')	Treatment of chronic lymphocytic leukaemia
Czech	Deoxyribosa phosphorothioat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Léčba chronické lymfatické leukemie
Danish	Fosforotidat deoxiribose (5'-tct-ccc-agc-gtg-cgc-cat-3')	Behandling af kronisk lymfocytær leukæmi
Dutch	Desoxyribose phosphorothioaat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Behandeling van chronische lymfocyttaire leukemie
Estonian	Deoksüriboos-fosforotioaat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Kroonilise lümfotsüüt leukeemia ravi
Finnish	Deoksiriboosi fosforotioaatti (5'-tct-ccc-agc-gtg-cgc-cat-3')	Kroonisen lymfosyyttileukemian hoito
French	désoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3')	Traitement de la leucémie lymphoïde chronique
German	Desoxyribosephosphorthioat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Behandlung der chronisch-lymphatischen Leukämie
Greek	Φωσφοροθειονικό άλας δεσοξυριβόζης (5'-tct-ccc-agc-gtg-cgc-cat-3')	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας
Hungarian	Dezoxiriboz foszforotioát (5'-tct-ccc-agc-gtg-cgc-cat-3')	Krónikus lymphoid leukémia kezelése
Italian	Desossiribosio fosforotioato (5'-tct-ccc-agc-gtg-cgc-cat-3')	Trattamento della leucemia linfocitica cronica
Latvian	Deoksiribozes fosfortioāts (5'-tct-ccc-agc-gtg-cgc-cat-3')	Hroniskas limfoleikozes ārstēšana
Lithuanian	Deoksiribozės fosforotioatas (5'-tct-ccc-agc-gtg-cgc-cat-3')	Lėtinės limfocitinės leukemijos gydymas
Polish	Deoksyrybozy fosforotioat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Leczenie przewlekłej białaczki limfocytarnej
Portuguese	desoxiribose fosforotidato (5'-tct-ccc-agc-gtg-cgc-cat-3')	Tratamento da leucemia linfocítica crónica
Slovak	Fosforotioát deoxyribózy (5' - tct-ccc-agc-gtg-cgc-cat-3')	Liečba chronickej lymfocytickej leukémie
Slovenian	Deoksiriboza fosforotioat (5'-tct-ccc-agc-gtg-cgc-car-3')	Zdravljenje kronične limfocitne levkemije
Spanish	Desoxi-ribosa fosforotioato (5'-tct-ccc-agc-gtg-cgc-cat-3')	Tratamiento de la leucemia linfocítica crónica
Swedish	Fosforotidat deoxiribos (5'-tct-ccc-agc-gtg-cgc-cat-3')	Behandling av kronisk lymfatisk leukemi

¹ At the time of transfer of sponsorship