



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 multiple myeloma

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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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2013 on request of the Sponsor.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/3/01/065) 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 multiple myeloma. The sponsorship was transferred to Aventis Pharma S.A. in December 2002 and then back to Voisin Consulting S.A.R.L. in August 2005.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called a plasma cell. Plasma cells are found in 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. Platelets make the blood clot, and white blood cells fight infection. In multiple myeloma an excessive number of plasma cells are produced. Normally, the division of cells takes place in a controlled manner but with multiple myeloma, the process gets out of control and abnormal plasma cells multiply, producing many myeloma cells. These fill up the bone marrow and interfere with production of the normal white cells, red cells and platelets. This leads to a number of possible complications, which include anaemia, bone pain and



fractures, raised levels of calcium in the blood and kidney disease. Multiple myeloma is life-threatening.

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

At the time of designation, multiple myeloma affected approximately 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 45,000 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?

The main treatment of multiple myeloma is chemotherapy (using drugs to kill cancer cells) usually combined with steroids (a group of chemical substances, the so-called hormones, which have an effect on the activity of certain organs). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation. Radiotherapy can be very useful to treat painful areas and weakened bones. Interferon alpha is a protein normally produced by the body during viral infections, such as flu. Sometimes a combination of interferon and chemotherapy may be used.

Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') could be of potential significant benefit for the treatment of multiple myeloma 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) prevents this process of cell death. The myeloma cells have too much of this protein and therefore the cells live longer than normal. Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') might prevent the formation B-cell lymphoma-associated protein 2, decrease the amount of it and might thereby restore the normal cell death process in the cancer cells.

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, one clinical trial in patients with multiple myeloma was ongoing.

Deoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3') was not marketed anywhere worldwide for multiple myeloma, 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).

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 multiple myeloma
Czech	Deoxyribosa phosphorothioat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Léčba mnohočetného myelomu
Danish	Fosforotidat deoxiribose (5'-tct-ccc-agc-gtg-cgc-cat-3')	Behandling af multipelt myelom
Dutch	Desoxyribose phosphorothioaat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Behandeling van multipel myeloom
Estonian	Deoksüriboos-fosforotioaat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Multiipelmüeloomi ravi
Finnish	Deoksiriboosi fosforotioaatti (5'-tct-ccc-agc-gtg-cgc-cat-3')	Multippelin myelooman hoito
French	désoxyribose phosphorothioate (5'-tct-ccc-agc-gtg-cgc-cat-3')	Traitement du myélome multiple
German	Desoxyribosephosphorthioat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Behandlung des multiplen Myeloms
Greek	Φωσφοροθειονικό άλας δεσοξυριβόζης (5'-tct-ccc-agc-gtg-cgc-cat-3')	θεραπεία του πολλαπλού μυελώματος
Hungarian	Dezoxiriboz foszforotioát (5'-tct-ccc-agc-gtg-cgc-cat-3')	Myeloma multiplex kezelése
Italian	Desossiribosio fosforotioato (5'-tct-ccc-agc-gtg-cgc-cat-3')	Trattamento del mieloma multiplo
Latvian	Deoksiribozes fosfortioāts (5'-tct-ccc-agc-gtg-cgc-cat-3')	Multiplās mielomas ārstēšana
Lithuanian	Deoksiribozės fosforotioatas (5'-tct-ccc-agc-gtg-cgc-cat-3')	Dauginės mielomos gydymas
Polish	Deoksyrybozy fosforotioat (5'-tct-ccc-agc-gtg-cgc-cat-3')	Leczenie szpiczaka mnogiego
Portuguese	desoxiribose fosforotidato (5'-tct-ccc-agc-gtg-cgc-cat-3')	Tratamento do mieloma múltiplo
Slovak	Fosforotioát deoxyribózy (5'-tct-ccc-agc-gtg-cgc-cat-3')	Liečba multiforného myelómu
Slovenian	Deoksiriboza fosforotioat (5'-tct-ccc-agc-gtg-cgc-car-3')	Zdravljenje multiplega mieloma
Spanish	Desoxi-ribosa fosforotioato (5'-tct-ccc-agc-gtg-cgc-cat-3')	Tratamiento del mieloma múltiple
Swedish	Fosforotidat deoxiribos (5'-tct-ccc-agc-gtg-cgc-cat-3')	Behandling av multipelt myelom

¹ At the time of transfer of sponsorship