



**COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

**PUBLIC SUMMARY OF  
POSITIVE OPINION FOR ORPHAN DESIGNATION  
OF**

**thiotepa**

**for the conditioning treatment prior to haematopoietic progenitor cell transplantation**

On 29 January 2007, orphan designation (EU/3/06/424) was granted by the European Commission to ADIENNE S.r.l, Italy, to thiotepa for the conditioning treatment prior to haematopoietic progenitor cell transplantation.

**What is conditioning treatment prior to haematopoietic progenitor cell transplantation?**

The term of “progenitor cell” is used to indicate those cells which are still immature, and do not express all the characteristics of the future mature cells which will derive from them. Haematopoietic progenitor cells are able to produce the cells of the blood (white blood cells, red blood cells), including the cells of the immune system and of the bone marrow. In some diseases it is necessary to give powerful drugs, which also destroy the haematopoietic progenitor cells in the bone marrow; these bone marrow cells then need to be replaced. In other diseases, the bone marrow or the immune system are absent, or working abnormally. In all these cases, it is sometimes appropriate to use a treatment called “haematopoietic progenitor cell transplantation”. This consists in replacing the abnormal cells of the immune system and the bone marrow of the patient by introducing new progenitor cells, generally from another person. Before the transplantation can take place, any existing bone marrow cells have to be eliminated from the patient. This is called “preparation” treatment or “conditioning” treatment. Diseases requiring such transplantation are life-threatening.

**What are the methods of conditioning treatment available?**

Available conditioning treatments are based on the use of chemotherapy (using drugs to destroy the cells) or radiotherapy (exposing the whole body to radiation to kill the cancer cells). Several treatments were authorised for the condition in the Community at the time of submission of the application for orphan drug designation. Thiotepa used in combination with other drugs might be of potential significant benefit for the conditioning treatment. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, conditioning treatment prior to haematopoietic progenitor cell transplantation was considered to concern about 28,000 persons in the European Union.

**How is this medicinal product expected to act?**

Thiotepa belongs to a group of medicines called alkylating agents. Alkylating agents are highly reactive chemicals that bind to substances in the cell, and can damage or kill the cells. It is thought that by using this mechanism, thiotepa could destroy the patient bone marrow before the transplantation of the new haematopoietic progenitor cells.

**What is the stage of development of this medicinal product?**

At the time of submission of the application for orphan designation, the effects of thiotepa had not been evaluated in experimental models. No clinical trials in patients with conditioning treatment prior to haematopoietic progenitor cell transplantation had been initiated.

Thiotepa was not authorised anywhere in the world for conditioning treatment prior to haematopoietic progenitor cell transplantation or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 6 December 2006 a positive opinion recommending the grant of the above-mentioned designation.

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Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

**For more information:**

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Patients' association contact point: Not available

\*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

**Translations of the active ingredient and indication in all EU languages  
and Norwegian and Icelandic**

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
English	Thiotepa	Conditioning treatment prior to haematopoietic progenitor cell transplantation
Bulgarian	Тиотепа	Кондициониращ режим преди трансплантация на хемopoетични стволови клетки
Czech	Thiotepa	Přípravná léčba před transplantací hematopoetických progenitorových buněk
Danish	Thiotepa	Konditionerende behandling før transplantation af hæmatopoietiske progenitorceller
Dutch	Thiotepa	Vorbereidende behandeling van een hematopoietische stamcellentransplantatie
Estonian	Tiotepa	Hematopoeetiliste eellasrakkude siirdamise ettevalmistav ravi
Finnish	Tiotepa	Esihoito ennen hematopoeettisten progenitorisolujen siirtoa
French	Thiotépa	Conditionnement précédant la greffe de cellules souches hématopoïétiques
German	Thiotepa	Zur Konditionierung vor einer hämatopoetischen Stammzelltransplantation
Greek	Θειοτέπα	Αγωγή προετοιμασίας πριν από μεταμόσχευση πρόγονων αιμοποιητικών κυττάρων
Hungarian	Thiotepa	Kondicionáló kezelés hematopoeitikus progenitor sejt transzplantációt megelőzően
Italian	Tiotepa	Trattamento di condizionamento precedente al trapianto di cellule progenitrici ematopoietiche
Latvian	Thiotepa	Premedikācija pirms hemopoētisko cilmjšūnu transplantācijas
Lithuanian	Tiotepa	Simptominis gydymas prieš kamieninių kraujodaros ląstelių transplantaciją
Polish	Tiotepa	Leczenie kondycjonujące przed przeszczepieniem macierzystych komórek krwiotwórczych
Portuguese	Tiotepa	Tratamento de acondicionamento precedente a transplante de células progenitoras hematopoieticas
Romanian	Tiotepa	Tratament de pregătire anterior transplantului de celule suşe hematopoeitice
Slovak	Tiotepa	Udržiavacia liečba pred transplantáciou hematopoetických progenitorových buniek
Slovenian	Tiotepa	Pripravljalno zdravljenje (kondicioniranje) pred transplantacijo krvotvornih zarodnih celic
Spanish	Tiotepa	Tratamiento de acondicionamiento previo al transplante de células progenitoras hematopoyéticas
Swedish	Tiotepa	Konsoliderande behandling inför stamcellstransplantation
Norwegian	Tiotepa	Kondisjonerende behandling før transplantasjon av hematopoietiske progenitorceller
Icelandic	Thiotepa	Undirbúningsmeðferð fyrir blóðmyndandi stofnfrumuígræðslu