European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF treosulfan for the conditioning treatment prior to haematopoietic progenitor cell transplantation

On 23 February 2004, orphan designation (EU/3/04/186) was granted by the European Commission to medac Gesellschaft fuer klinische Spezialpräparate mbH, Germany, for treosulfan 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 immune system and bone marrow. For diseases where the bone marrow or the immune system are absent, or working abnormally, or invaded by cancer cells, it is sometimes appropriate to use a treatment called haematopoietic progenitor cell transplantation**.** This consists of replacing the abnormal cells of the immune system and bone marrow, and introducing new progenitor cells, generally from another person ('donor'). Before the transplantation can take place, the abnormal cells and the immune cells that might react against the new donor cells have to be eliminated. 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). Several treatments were authorised for the condition in the Community at the time of submission of the application for orphan drug designation.

Treosulfan 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 27,000 persons in the European Union.

How is this medicinal product expected to act?
Treosulfan 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, treosulfan 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, clinical trials in patients with haematopoeitic progenitor cell transplantation were ongoing.

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Treosulfan was not marketed anywhere worldwide for conditioning treatment prior to haematopoeitic 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 14 January 2004 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.

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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition. 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

Language	Active Ingredient	Indication
English	Treosulfan	Conditioning treatment prior to haematopoietic progenitor cell transplantation
Danish	Treosulfan	Konditionerende behandling før transplantation af hæmatopoietiske progenitorceller
Dutch	Treosulfan	Voorbereidende behandeling van een hematopoietische stamcellentransplantatie
Finnish	Treosulfaani	Esihoito ennen hematopoieettisten progenitorisolujen siirtoa
French	Treosulfan	Conditionnement précédant la greffe de cellules souches hématopoïétiques
German	Treosulfan	Zur Konditionierung vor einer hämatopoetischen Stammzelltransplantation
Greek	Treosulfan	Αγωγή προετοιμασίας πριν από μεταμόσχευση πρόγονων αιμοποιητικών κυττάρων.
Italian	Treosulfan	Trattamento di condizionamento precedente al trapianto di cellule progenitrici ematopoietiche
Portuguese	Treosulfan	Tratamento de acondicionamento precedente a transplante de células progenitoras hematopoiéticas
Spanish	Treosulfán	Tratamiento de acondicionamiento previo al transplante de células progenitoras hematopoyéticas
Swedish	Treosulfan	Konsoliderande behandling inför stamcellstransplantation