



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

arsenic trioxide for the treatment of myelodysplastic syndromes

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in March 2010 on request of the sponsor.

On 29 March 2001, orphan designation EU/3/01/031 was granted by the European Commission to Cell Therapeutics (UK) Ltd., United Kingdom, for arsenic trioxide for the treatment of myelodysplastic syndromes.

The sponsorship was transferred to Cephalon UK Limited, United Kingdom, in April 2006 and subsequently to Cephalon Europe, France, in June 2007.

What are myelodysplastic syndromes?

Myelodysplastic syndromes are a distinct group of disorders in which the production of blood cells by the bone marrow is abnormal. The bone marrow is the spongy tissue found in the large bones. It has the function of making red cells (which are the main carriers of oxygen to body tissues), white blood cells (which fight infection), and platelets (which make the blood clot). In myelodysplastic syndromes their production is affected because these cells do not grow and mature normally. Consequently several symptoms can develop: fatigue or weakness (due to anaemia, the red cells deficit), infections (due to decrease in white blood cells) or easy bruising or abnormal bleeding (platelets deficit). Myelodysplastic syndromes are life threatening because they can result in severe anaemia, infections or haemorrhages and it can progress to acute leukaemia.

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

At the time of designation, myelodysplastic syndromes affected approximately 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 75,000 people, and is below the threshold 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).

*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 377,000,000 (Eurostat 2001) 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.



What treatments are available?

At the time of submission of the application for the orphan drug designation there was no treatment authorised in the European Union. Available therapeutic options for myelodysplastic syndromes included supportive care methods (e.g. antibiotics to treat infections, blood or platelet transfusions for anaemia or bleeding respectively), the use of products such as erythropoietin (a substance that stimulates the bone marrow to produce red cells), chemotherapy (using drugs that can kill the abnormal cells), and bone-marrow transplantation.

How is this medicine expected to work?

It is not completely understood how arsenic trioxide works in the human body. Among others arsenic trioxide breaks down the fundamental genetic material (DNA) of the cancer cells and thereby induces cell death.

What is the stage of development of this medicine?

The effects of arsenic trioxide were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with myelodysplastic syndromes were initiated.

Arsenic trioxide received orphan drug designation in the United States in 2000 for the treatment of myelodysplastic syndromes.

Arsenic trioxide was not marketed or designated as orphan medicinal product in the EU for the treatment of myelodysplastic syndromes, 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 9 February 2001 a positive opinion recommending the grant of the above-mentioned 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 European Union) 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

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Translations of the active ingredient and indication in all official EU languages¹

Language	Active ingredient	Indication
English	Arsenic trioxide	Treatment of myelodysplastic syndromes
Bulgarian	Арсенов триоксид	Лечение на миелодиспластични синдроми
Czech	Trioxid arsenitý	Léčba myelodysplastického syndromu
Danish	Arsenik trioxid	Behandling af myelodysplasisyndromer
Dutch	Arseentrioxide	Behandeling van de myelodysplastische syndromen
Estonian	Arseentrioksiid	Müelodüsplastiliste sündroomide ravi
Finnish	Arseenitrioksidi	Myelodysplastisten syndroomien hoito
French	Trioxyde d'arsenic	Traitement des syndromes myélodysplasiques
German	Arsentrioxid	Behandlung der Myelodysplasiesyndrome
Greek	Τριοξείδιο αρσενικού	Θεραπεία των συνδρόμων της μυελοδυσπλασίας
Hungarian	Arsenum trioxydatum	Myelodysplasias syndroma kezelése
Italian	Triossido di arsenico	Trattamento delle sindromi mielodisplastiche
Latvian	Arsēna trioksīds	Mielodisplastisko sindromu ārstēšana
Lithuanian	Arseno trioksidas	Mielodisplastinių sindromų gydymas
Maltese	Arsenic trioxide	Kura tas-sindromi mjelodisplastici
Polish	Arsenu tritlenek	Leczenie zespołów mielodysplastycznych
Portuguese	Trióxido de arsénico	Tratamento dos síndromes mielodisplásicos
Romanian	Trioxid de arsen	Tratamentul sindromului mielodisplazic
Slovak	Oxid arsenitý	Liečba myelodysplastického syndrómu
Slovenian	Arzenov trioksid	Zdravljenje mielodisplastičnega sindroma
Spanish	Trióxido de arsénico	Tratamiento de los síndromes mielodisplásticos
Swedish	Arseniktrioxid	Behandling av myelodysplasisyndrom

¹ At the time of transfer of sponsorship