



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

10 March 2010
EMA/COMP/95792/2008 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

arsenic trioxide for the treatment of acute myeloid leukaemia

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in February 2010 on request of the sponsor.

On 02 August 2007, orphan designation (EU/3/07/460) was granted by the European Commission to Cephalon Europe, France, for arsenic trioxide for the treatment of acute myeloid leukaemia.

What is acute myeloid leukaemia?

Acute myeloid leukaemia 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", which 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. In myeloid leukaemia, blasts that should develop into a type of white blood cells called granulocytes are affected. The blasts do not mature, and become too many. These blast cells are then found in the blood; they also accumulate in the bone marrow where they take the place of the other types of normal blood cells, causing anaemia, easy bruising, and frequent infections. Myeloid leukaemia can be acute, when it develops quickly with many blasts. Acute myeloid leukaemia is life-threatening.

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

At the time of designation, acute myeloid leukaemia affected less than 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 50,000 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: 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 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.



What treatments are available?

Treatment for leukaemia is complex, and depends on a number of factors including the type of leukaemia, the extent of the disease and whether the leukaemia has been treated before. It also depends on the age, the symptoms, and the general health of the patient. The primary treatment of acute myeloid leukaemia 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.

Arsenic trioxide could be of potential significant benefit for the treatment of acute myeloid leukaemia 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?

It is not completely understood how arsenic trioxide works in the human body. The sponsor proposes that arsenic trioxide breaks down the fundamental genetic material (DNA) of the cancer cells and thereby induces cancer 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, clinical trials in patients with acute myeloid leukaemia were partly completed.

Arsenic trioxide was not authorised anywhere worldwide for treatment of acute myeloid leukaemia. Orphan designation of arsenic trioxide was granted in the United States for treatment of acute myeloid leukaemia.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 27 June 2007 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, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Arsenic trioxide	Treatment of acute myeloid leukaemia
Bulgarian	Арсенов триоксид	Лечение на остра миелоидна левкемия
Czech	Trioxid arsenitý	Léčba akutní myeloidní leukémie
Danish	Arsenik trioxid	Behandling af akut myeloid leukæmi
Dutch	Arseentrioxide	Behandeling van acute myeloïde leukemie
Estonian	Arseentrioksiid	Akuutse müeloidse leukeemia ravi
Finnish	Arseenitrioksidi	Akuutin myelooisen leukemian hoito
French	Trioxyde d'arsenic	Traitement de la leucémie aiguë myéloïde
German	Arsentrioxid	Behandlung der akuten myeloischen Leukämie
Greek	Τριοξείδιο αρσενικού	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Arsenum trioxydatum	Akut myeloid leukaemia kezelése
Italian	Triossido di arsenico	Trattamento della leucemia mieloide acuta
Latvian	Arsēna trioksīds	Akūtas mieloleikozes ārstēšana
Lithuanian	Arseno trioksidas	Ūmios mieloleukozės gydymas
Maltese	Trioxid de arsen	Kura tal-lewkimja mjelojda akuta
Polish	Arsenu tritlenek	Leczenie ostrej białaczki szpikowej
Portuguese	Trióxido de arsénico	Tratamento da leucemia mielóide aguda
Romanian	Arsenic trioxide	Tratamentul leucemiei mieloide acute
Slovak	Oxid arzenitý	Liečba akútnej myeloickej leukémie
Slovenian	Arzenov trioksid	Zdravljenje akutne mieloične levkemije
Spanish	Trióxido de arsénico	Tratamiento de la leucemia mieloide aguda
Swedish	Arseniktrioxid	Behandling av akut myeloisk leukemi
Norwegian	Arsentrioksid	Behandling av akutt myelogen leukemi
Icelandic	Arsenik þríoxíð	Meðferð við bráðu kyrningahvítblæði

¹ At the time of designation