



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

2-chloro-9-[2-deoxy-2'-fluoro- β -D-arabinofuranosyl]adenine for the treatment of acute myeloid leukaemia

First publication	21 May 2003
Rev.1: administrative update	29 May 2007
Rev.2: administrative update	3 July 2007
Rev.3: transfer of sponsorship	18 August 2008
Rev.4: withdrawal from the Community Register	6 October 2014
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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2014 on request of the Sponsor.

On 8 May 2003, orphan designation (EU/3/03/141) was granted by the European Commission to Bioenvision Limited, United Kingdom, for 2-chloro-9-[2-deoxy-2'-fluoro- β -D-arabinofuranosyl]adenine for the treatment of acute myeloid leukaemia.

The sponsorship was transferred to Genzyme Europe BV, The Netherlands, in May 2008.

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" 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. 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 are developing into 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 and also accumulate in the bone marrow. 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 approximately 0.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 27,000 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?

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 leukemia is chemotherapy (using drugs to kill cancer cells) and currently there exist some drugs that can be used for the treatment of this condition. 2-chloro-9-[2-deoxy-2'-fluoro-β-D-arabinofuranosyl]adenine, also known as clofarabine, could be of potential significant benefit for the treatment of acute myeloid leukaemia. This is because it may act in a more effective way than other drugs. The 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?

Clofarabine is similar to a substance called adenine. Adenine is part of the fundamental genetic material of cells (DNA and RNA). Clofarabine can block the build up of genetic material and this might kill the cancer cells.

Clofarabine received orphan drug designation in the United States in 2002 for the treatment of primary refractory or relapsed acute myelogenous leukaemia in paediatric and adult patients.

What is the stage of development of this medicine?

The effects of clofarabine were evaluated in experimental models. Clinical studies with clofarabine in patients with acute myeloid leukaemia were ongoing at the time of submission of the application for orphan designation.

Clofarabine received orphan drug designation in the United States in 2002 for the treatment of primary refractory or relapsed acute myelogenous leukaemia in paediatric and adult patients.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 March 2003 recommending the granting of this designation.

*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 382,800,000 (Eurostat 2003).

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	2-chloro-9-[2-deoxy-2-fluoro-β-D-arabinofuranosyl]adenine	Treatment of acute myeloid leukaemia
Bulgarian	2-хлоро-9-[2-деокси-2-флуоро-β-D-арабинофуранозил]аденин	Лечение на остра миелоидна левкемия
Czech	2-chloro-9-[2-deoxy-2-fluoro-β-D-arabinofuranosyl]adenin	Léčba akutní myeloidní leukémie
Danish	2-chloro-9-[2-deoxy-2-fluoro-β-D-arabinofuranosyl]adenin	Behandling af akut myeloid leukæmi
Dutch	2-chloro-9-[2-deoxy-2-fluoro-β-D-arabinofuranosyl]adenine	Behandeling van acute myeloïde leukemie
Estonian	2-kloori-9-[2-desoksü-2-fluoro-β-D-arabinofuranosüül]adeniin	Akuutse müeloidse leukeemia ravi
Finnish	2-kloori-9-[2-deoksi-2-fluoro-β-D-arabinofuranosyyli]adeniini	Akuutin myelooisen leukemian hoito
French	2-chloro-9-[2-désoxy-2-fluoro-β-D-arabinofuranosyl]adénine	Traitement de la leucémie myéloïde aiguë
German	2-chlor-9-[2-deoxy-2-fluoro-β-D-arabinofuranosyl]adenin	Therapie der akuten myeloischen Leukämie
Greek	2-χλωρο-9-[2-δεοξυ- 2-φθορο- β- D-αραβινοφουρανοσυλ] αδενίνη	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	2-klór-9-[2-dezoxi-2-fluor-β-D-arabinofuranozil]-adenin	Akut myeloid leukaemia kezelése
Italian	2-cloro-9-[2-deossi-2-fluoro-β-D-arabinofuranosil]adenina	Trattamento della leucemia mieloide acuta
Latvian	2-hlor-9-[2-dezoksi-2-fluor-β-D-arabinofuranozil]adenīns	Akūtas mieloleikozes ārstēšana
Lithuanian	2-chlor-9-[2-deoksi-2-fluor-β-D-arabinofuranozil]adeninas	Ūmios mieloleukozės gydymas
Maltese	2-chloro-9-[2-deoxy-2-fluoro-β-D-arabinofuranosyl]adenine	Kura tal-lewkimja mjelojda akuta
Polish	2-chloro-9-[2-deoksy-2-fluoro-β-D-arabinofuranozylo]adenina	Leczenie ostrej białaczki szpikowej
Portuguese	2-cloro-9-[2-desoxi-2-fluoro-β-D-arabinofuranosil]adenina	Tratamento da leucemia mielóide aguda
Romanian	2-cloro-9-[2-deoxi-2-fluoro-β-D-arabinofuranozil]adenină	Tratamentul leucemiei mieloide acute
Slovak	2-chlór-9-[2-deoxy-2-fluór-β-D-arabinofuranozyl]adenín	Liečba akútnej myeloidkej leukémie
Slovenian	2-kloro-9-[2-deoksi-2-fluoro-β-D-arabinofuranozil]adenin	Zdravljenje akutne mieloične levkemije

¹ At the time of transfer of sponsorship

Language	Active ingredient	Indication
Spanish	2-cloro-9-[2-desoxi-2-fluoro-β-D-arabinofuranosil]adenina	Tratamiento de la leucemia mieloide aguda
Swedish	2-kloro-9-[2-deoxy-2-fluor-β-D-arabinofuranosyl]adenin	Behandling av akut myeloid leukemi