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

Document Date: London, 29 July 2008
Doc.Ref.: EMEA/COMP/221755/2005 Rev.2

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2008 on request of the sponsor.

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF troxacitabine for the treatment acute myeloid leukaemia

On 26 August 2005, orphan designation (EU/3/05/311) was granted by the European Commission to GMG BioBusiness United Kingdom, for troxacitabine for the treatment of acute myeloid leukaemia. The sponsorship was transferred to Mr Peter Hagger, United Kingdom, in March 2007.

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 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 and also accumulate in the bone marrow where they take the place of the other types of normal blood cells. When leukaemia develops quickly with many blasts it is called acute. Acute myeloid leukaemia is life-threatening.

What are the methods of treatment 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.

Troxacitabine 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.

7 Westferry Circus, Canary Wharf, London, E14 4HB, UK
Tel. (44-20) 74 18 84 00 Fax (44-20) 75 23 70 40
E-mail: orphandrugs@emea.europa.eu http://www.emea.europa.eu

What is the estimated number of patients affected by the condition*?
Based on the information provided by the sponsor and previous knowledge of the Committee, acute myeloid leukaemia was considered to affect approximately 0.7 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 32,000 persons.

How is this medicinal product expected to act?
Troxacitabine is similar to a group of substances called nucleosides. Natural nucleosides are part of the fundamental genetic material of the cell (DNA or RNA). According to the sponsor troxacitabine might block the build-up of genetic material and this might kill the cancer cells.

What is the stage of development of this medicinal product?
The effects of troxacitabine 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 ongoing.

Troxacitabine was not authorised anywhere worldwide for treatment of acute myeloid leukaemia, at the time of submission. Orphan designation of troxacitabine 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 13 July 2005 a positive opinion recommending the grant of the above-mentioned designation.

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:
Sponsor's contact details:
Mr Peter Hagger
Pharm Research Associates (UK) Limited
Pacific House
Imperial Way
Reading
RG2 0TD
United Kingdom
Telephone: +44 118 918 1125
Telefax: +44 118 918 1003
E-mail: HaggerPeter@praintl.com

* 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.

Patients' associations contact points:

Leukaemia Care
2 Shrubbery Avenue
Worcester
WR1 1QH
United Kingdom
0800 169 6680 (freephone for UK residents)
Telephone: +44 19 05 33 00 03 / + 44 84 57 67 32 03
Telefax: +44 19 05 33 00 90
E-mail: enquiries@leukaemiaCARE.org.uk

Ligue Nationale Contre le Cancer
13 Av. de la Grande Armee
75116 Paris
France
Telephone: +33 1 45 00 00 17
Tefefax: +33 1 45 00 63 06
E-mail: ligue@ligue-cancer.net

Aplastischen Anämie e. V.
Am Lettenholz 41
83646 Bad Tölz
Germany
Telephone: +49 80 41 80 65 06
Telefax : +49 80 41 80 65 07
E-mail: info@aplastische-anaemie.de

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

Language	Active Ingredient	Indication
English	Troxacitabine	Treatment of acute myeloid leukaemia
Czech	Troxacitabin	Léčba akutní myeloidní leukémie
Danish	Troxacitabin	Behandling af akut myeloid leukæmi
Dutch	Troxacitabine	Behandeling van acute myeloïde leukemie
Estonian	Troksatsitabiin	Akuutse müeloidse leukeemia ravi.
Finnish	Troksasitabiini	Akuutin myelooisen leukemian hoito
French	Troxacitabine	Traitement de la leucémie myéloïde aiguë
German	Troxacitabin	Behandlung der akuten myeloischen Leukämie
Greek	Troxacitabine	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Troxacitabin	Akut myeloid leukaemia kezelése
Italian	Troxacitabina	Trattamento della leucemia mieloide acuta
Latvian	Troksacitabīns	Akūtas mieloleikozes ārstēšana
Lithuanian	Troksacitabinas	Ūmios mieloleukozės gydymas
Polish	Troksacytabina	Leczenie ostrej białaczki szpikowej
Portuguese	Troxacitabina	Tratamento da leucemia mielóide aguda
Slovak	Troxacitabín	Liečba akútnej myeloickej leukémie
Slovenian	Troksacitabin	Zdravljenje akutne mieloične levkemije
Spanish	Troxacitabina	Tratamiento de la leucemia mieloide aguda
Swedish	Troxacitabin	Behandling av akut myeloisk leukemi
Norwegian	Troxacitabin	Behandling av akutt myelogen leukemi
Icelandic	Troxasítabín	Meðferð við bráðu kyrningahvítblæði