



Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of zosuquidar trihydrochloride for the treatment of acute myeloid leukaemia

On 17 February 2006, orphan designation (EU/3/06/355) was granted by the European Commission to Uppsala Medical Information System AB, Sweden, for zosuquidar trihydrochloride for the treatment of acute myeloid leukaemia.

The sponsorship was transferred to Kanisa Europe Limited, United Kingdom, in May 2006.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells. In this disease, the bone marrow produces large numbers of abnormal, immature white blood cells called 'blasts'. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is life-threatening because these immature cells take the place of the normal white blood cells. As a result, the patient's ability to fight diseases is reduced.

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 is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of 32,000 people.

What treatments are available?

Treatment for AML is complex and depends on a number of factors including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. The primary treatment for AML is chemotherapy (using medicines to kill cancer cells).

How is this medicine expected to work?

Cancer cells develop several mechanisms of resistance to evade the toxic effects of anticancer drugs. One of these mechanisms involves a protein called multidrug resistance (MDR) transporter. MDR pumps substances out of the cell. In fact, MDR can pump anticancer agents out of the cancer cells. Therefore, the drugs cannot reach their target within the cancer cells and do not kill them. Zosuquidar does not have anticancer properties per se. However, it is an inhibitor of the MDR transporter and by blocking this transporter it is expected to help anticancer drugs to overcome this resistance mechanism and to exert their activity.

* 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 Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

What is the stage of development of this medicine?

The effects of zosuquidar 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.

Zosuquidar thihydrochloride was not authorised anywhere worldwide for acute myeloid leukaemia 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 11 January 2006 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information:

Sponsor's contact details:

Kanisa Europe Limited

Mofo Notices Limited

C/Morrison & Foerster MNP

City Point, One Ropemaker Street

London, EC2Y 9AW

United Kingdom

Telephone: +44 207 920 4081

Telefax: +44 207 496 8581

E-mail: MODonnell@mofo.com

Patients' associations contact points:

Leukaemia CARE

One Birch Court
Blackpole East
Worcester
Worcestershire WR3 8SG
United Kingdom
Telephone: +44 1905 75 59 77 / +44 845 521 34 56
24 hour CARE line 0800 169 6680 (UK residents only)
Telefax: +44 1905 755166
E-mail: info@leukaemiacare.org.uk

Ligue Nationale Contre le Cancer

14 Rue Corvisart
75013 Paris
France
Telephone: +33 1 53 55 24 00
Telefax: +33 1 43 36 91 10
E-mail: ligue@ligue-cancer.net

Deutsche Krebshilfe e. V.

Buschstr. 32
53113 Bonn
Germany
Telephone: +49 2 287 29 900
Telefax: +49 2 287 29 90 11
E-mail: deutsche@krebshilfe.de

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

Language	Active Ingredient	Indication
English	Zosuquidar trihydrochloride	Treatment of acute myeloid leukaemia
Czech	Zosuchidar trihydrochlorid	Léčba akutní myeloidní leukémie
Danish	Zosuquidartrihydrochlorid	Behandling af akut myeloid leukæmi
Dutch	Zosuquidartrihydrochloride	Behandeling van acute myeloïde leukemie
Estonian	Zosukidaartrivesinikkloriid	Akuutse müeloidse leukeemia ravi
Finnish	Tsosukidaartrihydrokloridi	Akuutin myelooisen leukemian hoito
French	Trihydrochlorure de zosuquidar	Traitement de la leucémie aiguë myéloïde
German	Zosuquidar trihydrochlorid	Behandlung der akuten myeloischen Leukämie
Greek	Ζοσουκουιδάρη, τριϋδροχλωρική	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Zosuquidar trihidroklorid	Akut myeloid leukaemia kezelése
Italian	Triidrocloruro di zosuquidar	Trattamento della leucemia mieloide acuta
Latvian	Zosukvidāra trihidrohlorīds	Akūtas mieloleikozes ārstēšana
Lithuanian	Zosukvidaro trihidrochloridas	Ūmios mieloleukozės gydymas
Polish	Trójchlorowodorek zosuchidaru	Leczenie ostrej białaczki szpikowej
Portuguese	Tricloridrato de zosuquidar	Tratamento da leucemia mieloide aguda
Slovak	Zosuchidar trihydrochlorid	Liečba akútnej myeloickej leukémie
Slovenian	Zosukvidar trihidroklorid	Zdravljenje akutne mieloične levkemije
Spanish	Triclorhidrato de zosuquidar	Tratamiento de la leucemia mieloide aguda
Swedish	Zosuquidartrihydroklorid	Behandling av akut myeloisk leukemi
Norwegian	Zosukvidartrihydroklorid	Behandling av akutt myelogen leukemi
Icelandic	Zósúquíðar þriháðróklóríð	Meðferð við bráðu kyrningahvítblæði