



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

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Committee for Orphan Medicinal Products

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

On 10 July 2008, orphan designation (EU/3/08/558) was granted by the European Commission to Cyclacel Limited, United Kingdom, for sapacitabine for the treatment of acute myeloid leukaemia.

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 1 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 around 50,000 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). Satisfactory argumentation has been submitted by the sponsor to justify the assumption that sapacitabine might be of potential significant benefit for the treatment of acute myeloid leukaemia because it might improve the long-term outcome of the patients and because it is planned to be administered as capsules. These assumptions will need to be confirmed at the time of marketing authorisation, to maintain the orphan status.

How is this medicine expected to work?

Sapacitabine is expected to kill dividing cells. Sapacitabine is a nucleoside analogue, which means that it is similar to a nucleoside, a chemical that forms part of the DNA (the fundamental genetic material in cells). In the body, sapacitabine is expected to interfere with an enzyme called DNA polymerase, which is involved in the formation of DNA. This is expected to slow down the production of DNA and the growth of the cancerous cells.

* 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 Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

What is the stage of development of this medicine?

The effects of sapacitabine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with acute myeloid leukaemia had been started.

At the time of submission, sapacitabine was not authorised anywhere in the world for AML or designated as orphan medicinal product elsewhere for this condition.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted a positive opinion on 14 May 2008, recommending the granting 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.

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

Language	Active Ingredient	Indication
English	Sapacitabine	Treatment of acute myeloid leukaemia
Bulgarian	Сапацитабин	Лечение на остра миелоидна левкемия
Czech	Sapacitabine	Léčba akutní myeloidní leukémie
Danish	Sapacitabine	Behandling af akut myeloid leukæmi
Dutch	Sapacitabine	Behandeling van acute myeloïde leukemie
Estonian	Sapatsitabiin	Akuutse müeloidse leukeemia ravi
Finnish	Sapacitabine	Akuutin myelooisen leukemian hoito
French	Sapacitabine	Traitement de la leucémie aiguë myéloïde
German	Sapacitabine	Behandlung der akuten myeloischen Leukämie
Greek	Sapacitabine (Σαπασιταμπίνη)	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Sapacitabine	Akut myeloid leukaemia kezelése
Italian	Sapacitabina	Trattamento della leucemia mieloide acuta
Latvian	Sapacitabīns	Akūtas mieloleikozes ārstēšana
Lithuanian	Sapacitabinas	Ūmios mieloleukozės gydymas
Maltese	Sapacitabine	Kura tal-lewkimja mjelojda akuta
Polish	Sapacitabine	Leczenie ostrej białaczki szpikowej
Portuguese	Sapacitabina	Tratamento da leucemia mielóide aguda
Romanian	Sapacitabină	Tratamentul leucemiei mieloide acute
Slovak	Sapacitabín	Liečba akútnej myeloickej leukémie
Slovenian	Sapacitabin	Zdravljenje akutne mieloične levkemije
Spanish	Sapacitabina	Tratamiento de la leucemia mieloide aguda
Swedish	Sapacitabine	Behandling av akut myeloisk leukemi
Norwegian	Sapacitabin	Behandling av akutt myelogen leukemi
Icelandic	Sapacítabín	Meðferð við bráðu kyrningahvítblæði