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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Azacitidine for the treatment of acute myeloid leukaemia

On 29 November 2007, orphan designation (EU/3/07/509) was granted by the European Commission to Pharmion Ltd, United Kingdom, for azacitidine for the treatment of acute myeloid leukaemia.

The sponsorship was transferred to Celgene Europe Limited, United Kingdom, in October 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 of 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 leakaemia affected less than 2 people in 10,000 per year in the European Union (EU)*. This is equivalent to a total of fewer than100,000 people per year, which was considered to be below the threshold for orphan designation. This is based on the information provided by the sponsor and 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 on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 498,000,000 (Eurostat 2006).

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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.

Azacitidine 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?

Azacitidine is similar to cytidine, which in turn is a building block of the fundamental genetic material of cells (DNA and RNA). Azacitidine is used by the cells instead of cytidine and the result is a block in the synthesis of DNA and RNA, and a decrease in the growth of tumour cells.

What is the stage of development of this medicine?

The effects of azacitidine 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.

Azacitidine was not authorised anywhere in the world for the treatment of acute myeloid leukaemia, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 October 2007 recommending the granting of this designation.

Update: Azacitidine (Vidaza) has been authorised in the EU since 17 December 2008 for the treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with:

- intermediate-2 and high-risk myelodysplastic syndromes (MDS) according to the International Prognostic Scoring System (IPSS),
- chronic myelomonocytic leukaemia (CMML) with 10-29 % marrow blasts without myeloproliferative disorder,
- acute myeloid leukaemia (AML) with 20-30 % blasts and multi-lineage dysplasia, according to World Health Organisation (WHO) classification.

More information on Vidaza can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

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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 languages[1], Norwegian and Icelandic

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

[1] At the time of designation