EMA/COMP/137787/2006 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Decitabine for the treatment of acute myeloid leukaemia

First publication	18 July 2007
Rev.1: information about Marketing Authorisation	11 September 2013

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.

On 8 June 2006, orphan designation (EU/3/06/370) was granted by the European Commission to MGI Pharma Limited, United Kingdom, for decitabine for the treatment of acute myeloid leukaemia.

The sponsorship was transferred to Janssen-Cilag International NV, Belgium, 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 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 less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 47,000 people*, and is below the

*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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,200,000 (Eurostat 2013).

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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 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.

Decitabine might be of potential significant benefit for the treatment of acute myeloid leukaemia, because it may act in a different way than other medicines, which might potentially be easier to use in the older patient population. 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?

Decitabine is a chemical substance, which is related to cytidine. Cytidine is part of the fundamental genetic material of cells (DNA and RNA). Decitabine blocks (inhibits) the building up (synthesis) of DNA and thereby could inhibit the growth of tumour cells.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with acute myeloid leukaemia were ongoing.

Decitabine was not authorised anywhere worldwide for acute myeloid leukaemia, at the time of submission.

Orphan designation of decitabine was granted in the European Union and in the United States for treatment of myelodysplastic syndromes.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 April 2006 recommending the granting of this designation.

Update: Decitabine (Dacogen) has been authorised in the EU since 20 September 2012 for treatment of adult patients aged 65 years and above with newly diagnosed de novo or secondary acute myeloid leukaemia (AML), according to the World Health Organization (WHO) classification, who are not candidates for standard induction chemotherapy.

More information on Dacogen 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

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	Decitabine	Treatment of acute myeloid leukaemia
Bulgarian	Децитабин	Лечение на остра миелоидна левкемия
Czech	Decitabin	Léčba akutní myeloidní leukémie
Danish	Decitabin	Behandling af akut myeloid leukæmi
Dutch	Decitabine	Behandeling van acute myeloïde leukemie
Estonian	Detsitabiin	Akuutse müeloidse leukeemia ravi
Finnish	Desitabiini	Akuutin myelooisen leukemian hoito
French	Décitabine	Traitement de la leucémie aiguë myéloïde
German	Decitabine	Behandlung der akuten myeloischen Leukämie
Greek	Decitabine	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Decitabin	Akut myeloid leukaemia kezelése
Italian	Decitabina	Trattamento della leucemia mieloide acuta
Latvian	Decitabīns	Akūtas mieloleikozes ārstēšana
Lithuanian	Decitabinas	Ūmios mieloleukozės gydymas
Polish	Decytabina	Leczenie ostrej białaczki szpikowej
Portuguese	Decitabina	Tratamento da leucemia mielóide aguda
Romanian	Decitabină	Tratamentul leucemiei mieloide acute
Slovak	Decitabín	Liečba akútnej myeloickej leukémie
Slovenian	Decitabin	Zdravljenje akutne mieloične levkemije
Spanish	Decitabina	Tratamiento de la leucemia mieloide aguda
Swedish	Decitabin	Behandling av akut myeloisk leukemi
Norwegian	Decitabin	Behandling av akutt myelogen leukemi
Icelandic	Desítabín	Meðferð við bráðu kyrningahvítblæði

[1] At the time of transfer of sponsorship