30 March 2015
EMA/COMP/24067/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Alvocidib for the treatment of acute myeloid leukaemia

On 12 February 2015, orphan designation (EU/3/15/1437) was granted by the European Commission to Theorem Clinical Research GmbH, Germany, for alvocidib for the treatment of acute myeloid leukaemia.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (cells that fight against infections). In patients with AML, the bone marrow (the spongy tissue inside the large bones, where blood cells are produced) produces large numbers of abnormal, immature white blood cells. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is a long-term debilitating and life-threatening disease because these abnormal immature cells take the place of the normal blood cells, causing bleeding episodes, blood clots and reducing the patient's ability to fight infections.

What is the estimated number of patients affected by the condition?

At the time of designation, AML affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 people[*], and is below the 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 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. At the time of designation, the main treatments for AML were chemotherapy (medicines to treat cancer) and haematopoietic (blood) stem-cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow).

[*]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 512,900,000 (Eurostat 2015).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that alvocidib might be of significant benefit for patients with AML because initial studies suggest that it can improve responses when added to authorised treatments, including in patients whose disease has come back after previous treatment. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Alvocidib is a medicine that blocks the action of enzymes in the body called 'cyclin-dependent kinases' that are involved in the control of cell division (needed to produce new cells), as well as in the production of new proteins. By blocking the action of these enzymes, alvocidib can prevent leukaemia cells from dividing, eventually resulting in the death of the cell. In addition, if given before other cancer treatments that act at a particular stage of cell division, it can synchronise the cancerous cells so that they are in the most vulnerable stage at time other cancer treatments are given, maximising the effect of treatment.

What is the stage of development of this medicine?

The effects of alvocidib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with alvocidib in patients with AML were ongoing.

At the time of submission, alvocidib was not authorised anywhere in the EU for AML. Orphan designation of alvocidib had been granted in the United States for treatment of this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2015 recommending the granting of this 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;
- 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:

Theorem Clinical Research GmbH
Königsteiner Strasse 10
65812 Bad Soden a.Ts.
Germany
Tel. +49 6135 7063 534
Fax +49 6196 5228 0
E-mail: Evanthia.fritz@theoremclinical.com

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	Alvocidib	Treatment of acute myeloid leukaemia
Bulgarian	Алвоцидиб	Лечение на остра миелоидна левкемия
Croatian	Alvocidib	Liječenje akutne mijeloične leukemije
Czech	Alvocidib	Léčba akutní myeloidní leukémie
Danish	Alvocidib	Behandling af akut myeloid leukæmi
Dutch	Alvocidib	Behandeling van acute myeloïde leukemie
Estonian	Alvotsidiib	Akuutse müeloidse leukeemia ravi
Finnish	Alvosidibi	Akuutin myelooisen leukemian hoito
French	Alvocidib	Traitement de la leucémie aiguë myéloïde
German	Alvocidib	Behandlung der akuten myeloischen Leukämie
Greek	Αλβοσιτίμπη	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Alvocidib	Akut myeloid leukaemia kezelése
Italian	Alvocidib	Trattamento della leucemia mieloide acuta
Latvian	Alvocidibs	Akūtas mieloleikozes ārstēšana
Lithuanian	Alvocidibas	Ūmios mieloleukozės gydymas
Maltese	Alvocidib	Kura tal-lewkimja mjelojda akuta
Polish	Alwocydyb	Leczenie ostrej białaczki szpikowej
Portuguese	Alvocidib	Tratamento da leucémia mielóide aguda
Romanian	Alvocidib	Tratamentul leucemiei mieloide acute
Slovak	Alvocidib	Liečba akútnej myeloickej leukémie
Slovenian	Alvocidib	Zdravljenje akutne mieloične levkemije
Spanish	Alvocidib	Tratamiento de la leucemia mieloide aguda
Swedish	Alvocidib	Behandling av akut myeloisk leukemi
Norwegian	Alvocidib	Behandling av akutt myelogen leukemi
Icelandic	Alvócidíb	Meðferð við bráðu kyrningahvítblæði

[1] At the time of designation