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EMA/COMP/93562/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Volasertib for the treatment of acute myeloid leukaemia

On 26 March 2014, orphan designation (EU/3/14/1255) was granted by the European Commission to Boehringer Ingelheim International GmbH, Germany, for volasertib 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 white blood cells, reducing the patient's ability to fight infections.

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 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 511,100,000 (Eurostat 2014).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with AML because early clinical studies showed that it might prolong patients' survival when used in combination with existing treatments. 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?

Volasertib is a small molecule that is expected to block the activity of an enzyme called 'polo-like kinase' (PLK1), which is involved in cell division. By blocking this enzyme, the medicine is expected to prevent the abnormal division of cancer cells in AML patients, thus preventing the growth of the cancer.

What is the stage of development of this medicine?

The effects of volasertib have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2014 recommending the granting of this designation.

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

[1] At the time of designation