6 January 2017
EMA/741003/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ivosidenib for the treatment of acute myeloid leukaemia

On 12 December 2016, orphan designation (EU/3/16/1802) was granted by the European Commission to QRC Consultants Ltd, United Kingdom, for ivosidenib 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 a reduced ability to fight infections.

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

At the time of designation, AML affected approximately 1.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 57,000 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 procedure where the patient's bone marrow is cleared of cells and replaced by stem cells to form new bone marrow that produces healthy blood cells).

*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 513,700,000 (Eurostat 2016).

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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 studies have shown benefit in AML patients who cannot be treated using available medicines or whose disease has returned after 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?

Around a sixth of patients with AML have a fault in a gene called *IDH1*, which causes production of an abnormal IDH1 protein. The abnormal protein makes a chemical (2-hydroxyglutarate) that causes cells to become cancerous. Ivosidenib is expected to block the activity of the abnormal IDH1 protein and so reduces production of 2-hydorxyglutarate thereby preventing formation of cancer cells.

What is the stage of development of this medicine?

The effects of ivosidenib have been evaluated in experimental models.

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

At the time of submission, ivosidenib was not authorised anywhere in the EU for AML. Orphan designation of ivosidenib had been granted in the US for AML.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 November 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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

[1] At the time of designation