



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Crenolanib besylate for the treatment of acute myeloid leukaemia

On 14 October 2016, orphan designation (EU/3/16/1748) was granted by the European Commission to Arog Pharmaceuticals Europe Ltd, Ireland, for crenolanib besylate 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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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 (where the patient's bone marrow is replaced by stem cells to form new bone marrow that can produce 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).



The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with AML, because early data showed that there was no evidence of disease after treatment with the medicine in patients whose disease did not respond to other treatments or had come back. 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?

Crenolanib besylate is a 'tyrosine-kinase inhibitor'. This means that it blocks the activity of enzymes known as tyrosine kinases found in certain receptors in cancer cells. This medicine blocks the FLT3 receptor, which is involved in stimulating the AML cells to divide uncontrollably. By blocking this receptor, crenolanib besylate is expected to stop cell division and lead to cell death, and thus slow down the progression of the disease.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 2016 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:

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Crenolanib besylate	Treatment of acute myeloid leukaemia
Bulgarian	Креноланиб безилат	Лечение на остра миелоидна левкемия
Croatian	Crenolanib besilat	Liječenje akutne mijeloične leukemije
Czech	Crenolanib besilat	Léčba akutní myeloidní leukémie
Danish	Crenolanib besilat	Behandling af akut myeloid leukæmi
Dutch	Crenolanib besilaat	Behandeling van acute myeloïde leukemie
Estonian	Crenolanib besülaat	Akuutse müeloidse leukeemia ravi
Finnish	Krenolanibi besilaatti	Akuutin myelooisen leukemian hoito
French	Crenolanib bésylate	Traitement de la leucémie aiguë myéloïde
German	Crenolanib besilat	Behandlung der akuten myeloischen Leukämie
Greek	Κρενολανίμπη βεσυλική	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Crenolanib bezilát	Akut myeloid leukaemia kezelése
Italian	Crenolanib besilato	Trattamento della leucemia mieloide acuta
Latvian	Krenolaniba besilāts	Akūtas mieloleikozes ārstēšana
Lithuanian	Krenolanibo besilatas	Ūmios mieloleukozės gydymas
Maltese	Crenolanib besylate	Kura tal-lewkimja mjelojda akuta
Polish	Krenolanib bezylanu	Leczenie ostrej białaczki szpikowej
Portuguese	Besilato de crenolanibe	Tratamento da leucémia mielóide aguda
Romanian	Crenolanib besilat	Tratamentul leucemiei mieloide acute
Slovak	Crenolanib besilát	Liečba akútnej myeloickej leukémie
Slovenian	Krenolanib bezilat	Zdravljenje akutne mieloične levkemije
Spanish	Besilato de crenolanib	Tratamiento de la leucemia mieloide aguda
Swedish	Crenolanib besilat	Behandling av akut myeloisk leukemi
Norwegian	Krenolanibbesilat	Behandling av akutt myelogen leukemi
Icelandic	Crenólanib besýlat	Meðferð við bráðu kyrningahvítblæði

¹ At the time of designation