



EUROPEAN MEDICINES AGENCY
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Public summary of opinion on orphan designation

Pracinostat for the treatment of acute myeloid leukaemia

On 16 October 2017, orphan designation (EU/3/17/1927) was granted by the European Commission to Helsinn Birex Pharmaceuticals Ltd, Ireland, for pracinostat 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 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 515,700,000 (Eurostat 2017).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with AML because studies show that adding pracinostat to standard treatment in elderly patients improved disease control and survival (the time patients lived). 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?

The medicine, pracinostat, blocks the activity of enzymes called histone deacetylases (HDACs), which are involved in switching the activity of genes on and off within cells. In AML, pracinostat is expected to help keep genes that suppress the division and growth of the cancer cells switched 'on'. This is expected to stop the cancer cells from multiplying and to activate processes that kill the cell, thereby slowing down the growth of the cancer.

What is the stage of development of this medicine?

The effects of pracinostat have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with pracinostat in patients with AML was ongoing.

At the time of submission, pracinostat 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 7 September 2017 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	Pracinostat	Treatment of acute myeloid leukaemia
Bulgarian	ПрациноSTAT	Лечение на остра миелоидна левкемия
Croatian	Pracinostat	Liječenje akutne mijeloične leukemije
Czech	Pracinostat	Léčba akutní myeloidní leukémie
Danish	Pracinostat	Behandling af akut myeloid leukæmi
Dutch	Pracinostat	Behandeling van acute myeloïde leukemie
Estonian	Pratsinostaat	Akuutse müeloidse leukeemia ravi
Finnish	Prasinostaatti	Akuutin myelooisen leukemian hoito
French	Pracinostat	Traitement de la leucémie aiguë myéloïde
German	Pracinostat	Behandlung der akuten myeloischen Leukämie
Greek	Πρακινოსτάτη	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Pracinosztát	Akut myeloid leukaemia kezelése
Italian	Pracinostat	Trattamento della leucemia mieloide acuta
Latvian	Pracinostats	Akūtas mieloleikozes ārstēšana
Lithuanian	Pracinostatas	Ūmios mieloleukozės gydymas
Maltese	Pracinostat	Kura tal-lewkimja mjelojda akuta
Polish	Pracynostat	Leczenie ostrej białaczki szpikowej
Portuguese	Pracinostate	Tratamento da leucémia mielóide aguda
Romanian	Pracinostat	Tratamentul leucemiei mieloide acute
Slovak	Pracinostat	Liečba akútnej myeloickej leukémie
Slovenian	Pracinostat	Zdravljenje akutne mieloične levkemije
Spanish	Pracinostat	Tratamiento de la leucemia mieloide aguda
Swedish	Prakinostat	Behandling av akut myeloisk leukemi
Norwegian	Prakinostat	Behandling av akutt myelogen leukemi
Icelandic	Prakínóstat	Meðferð við bráðu kyrningahvítblæði

¹ At the time of designation