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Public summary of opinion on orphan designation

Pevonedistat for the treatment of acute myeloid leukaemia

On 25 July 2019, orphan designation EU/3/19/2186 was granted by the European Commission to Takeda Pharma A/S, Denmark, for pevonedistat for the treatment of acute myeloid leukaemia.

What is acute myeloid leukemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (cells that fight infection). In patients with AML, the bone marrow (the spongy tissue inside the large bones, where blood cells are produced) produces 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 the abnormal immune cells take the place of the normal blood cells, causing bleeding episodes, blood clots and reduced ability to fight infections.

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

At the time of designation, AML affected less than 1.8 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 93,000 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 depends on several 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 518,400,000 (Eurostat 2019).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with AML. Early studies indicate that adding pevonedistat to another cancer medicine azacitidine could improve survival of patients who had not been previously treated for their AML.

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?

Pevonedistat blocks the activity of an enzyme in the body called NEDD8-activating enzyme (NAE). NAE is involved in the growth and spread of cancer cells. By blocking NAE in patients with AML, pevonedistat is expected to prevent development and worsening of cancer.

What is the stage of development of this medicine?

The effects of pevonedistat have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 June 2019, 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.

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

[1] At the time of designation