



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Ulocuplumab for the treatment of acute myeloid leukaemia

On 12 February 2015, orphan designation (EU/3/15/1445) was granted by the European Commission to Bristol-Myers Squibb Pharma EEIG, United Kingdom, for ulocuplumab 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 reducing the patient's 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 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).

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\*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 512,900,000 (Eurostat 2015).



The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with AML because early studies suggest that, following treatment with ulocuplumab in combination with other chemotherapy medicines, patients whose disease had come back or did not respond to previous treatment remained without symptoms for a longer period than what has been reported in studies with other chemotherapy medicines. 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?**

Ulocuplumab is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a protein called chemokine receptor type 4 (CXCR4). This protein is found at high levels on the surface of AML cells, and it is thought to play a role in the growth and survival of the cancer. When ulocuplumab attaches to CXCR4, it is expected to block the growth of the AML cells, thus slowing down the progression of the disease.

### **What is the stage of development of this medicine?**

The effects of ulocuplumab have been evaluated in experimental models.

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

At the time of submission, ulocuplumab 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 9 January 2015 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<sup>1</sup>, Norwegian and Icelandic

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

<sup>1</sup> At the time of designation