19 September 2019
EMA/452780/2018

Public summary of opinion on orphan designation

Tamibarotene for the treatment of acute myeloid leukaemia

On 31 July 2018, orphan designation (EU/3/18/2053) was granted by the European Commission to Lakeside Regulatory Consulting Services Ltd, United Kingdom, for tamibarotene 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 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 1.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 72,000 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 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 517,400,000 (Eurostat 2018).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with AML. Laboratory data and early results in patients who cannot receive intensive chemotherapy suggest that it can have positive effects when combined with existing treatments. 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?

Tamibarotene is a 'synthetic retinoid' (a substance that is related to vitamin A). Tamibarotene is expected to work by attaching to a target in cells called the retinoic acid receptor alpha, which plays an important role in the growth of white blood cells. Patients with AML have more of this receptor in the cell and not enough natural retinoids to stimulate the growth of white blood cells. Tamibarotene is expected to work in the same way as retinoids, stimulating the abnormal, immature white blood cells to mature into normal white blood cells. This in turn is expected to control the growth of the cancer and reduce its symptoms.

What is the stage of development of this medicine?

The effects of tamibarotene 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, tamibarotene was authorised in Japan for the treatment of a specific type of AML called acute promyelocytic leukaemia.

At the time of submission, the medicine was not authorised anywhere in the EU for AML. Orphan designation of tamibarotene 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 21 June 2018 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 languages[1], Norwegian and Icelandic

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

[1] At the time of designation