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Public summary of opinion on orphan designation

Gilteritinib for the treatment of acute myeloid leukaemia

On 17 January 2018, orphan designation (EU/3/17/1961) was granted by the European Commission to Astellas Pharma Europe B.V., the Netherlands, for gilteritinib (also known as ASP2215) 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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 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).

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 because preliminary results showed that patients whose AML was previously treated but had come back or did not respond to previous treatments, responded to treatment with gilteritinib. At the time of designation there were no other treatment options available for such patients.

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?

Gilteritinib blocks the action of enzymes known as tyrosine kinases, in particular a tyrosine kinase called FLT3. FLT3 can be found on the surface of cancer cells in AML and is involved in stimulating the cells to multiply uncontrollably. By blocking FLT3, gilteritinib is expected to stop cell growth and lead to cell death, and thus slow down the development of the disease.

What is the stage of development of this medicine?

The effects of gilteritinib have been evaluated in experimental models.

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

At the time of submission, gilteritinib was not authorised anywhere in the EU for AML. Orphan designation of gilteritinib 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 December 2017 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:

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

[1] At the time of designation