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EMA/COMP/72835/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Venetoclax for the treatment of acute myeloid leukaemia

On 17 February 2016, orphan designation (EU/3/16/1617) was granted by the European Commission to Abbvie Ltd., United Kingdom, for venetoclax 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 approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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).

^{*}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 513,700,000 (Eurostat 2016).



The sponsor has provided sufficient information to show that venetoclax might be of significant benefit for patients with AML because early studies show that, when used in combination with other medicines, it can produce a response in the disease in patients older than 65 years who cannot receive standard treatment. 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?

Venetoclax is expected to work by blocking proteins called Bcl-2. These proteins allow cells to stay alive by preventing the natural process that leads to cell death (apoptosis). Bcl-2 proteins can be found in high levels in cancer cells. By blocking the action of these proteins, the medicine is expected to make cancer cells more responsive to this natural process, causing their death and slowing the growth of the cancer. As Bcl-2 proteins have been shown to be associated with resistance to conventional chemotherapy, this medicine may also reduce resistance and allow other medicines to be more effective in their treatment.

What is the stage of development of this medicine?

The effects of venetoclax have been evaluated in experimental models.

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

At the time of submission, venetoclax 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 21 January 2016 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	Venetoclax	Treatment of acute myeloid leukaemia
Bulgarian	Венетоклакс	Лечение на остра миелоидна левкемия
Croatian	Venetoklaks	Liječenje akutne mijeloične leukemije
Czech	Venetoclaxum	Léčba akutní myeloidní leukémie
Danish	Venetoclax	Behandling af akut myeloid leukæmi
Dutch	Venetoclax	Behandeling van acute myeloïde leukemie
Estonian	Venetoklaks	Akuutse müeloidse leukeemia ravi
Finnish	Venetoklaksi	Akuutin myelooisen leukemian hoito
French	Venetoclax	Traitement de la leucémie aiguë myéloïde
German	Venetoclax	Behandlung der akuten myeloischen Leukämie
Greek	Βενετοκλάξη	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Venetoklax	Akut myeloid leukaemia kezelése
Italian	Venetoclax	Trattamento della leucemia mieloide acuta
Latvian	Venetoklaks	Akūtas mieloleikozes ārstēšana
Lithuanian	Venetoklaksas	Ūmios mieloleukozės gydymas
Maltese	Venetoclax	Kura tal-lewkimja mjelojda akuta
Polish	Wenetoklaks	Leczenie ostrej białaczki szpikowej
Portuguese	Venetoclax	Tratamento da leucémia mielóide aguda
Romanian	Venetoclax	Tratamentul leucemiei mieloide acute
Slovak	Venetoklax	Liečba akútnej myeloickej leukémie
Slovenian	Venetoklaks	Zdravljenje akutne mieloične levkemije
Spanish	Venetoclax	Tratamiento de la leucemia mieloide aguda
Swedish	Venetoclax	Behandling av akut myeloisk leukemi
Norwegian	Venetoclax	Behandling av akutt myelogen leukemi
Icelandic	Venetóklax	Meðferð við bráðu kyrningahvítblæði

¹ At the time of designation