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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Gemtuzumab ozogamicin for the treatment of acute myeloid leukaemia

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Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 18 October 2000, orphan designation (EU/3/00/005) was granted by the European Commission to Wyeth Europa Limited, United Kingdom, for gemtuzumab ozogamicin for the treatment of acute myeloid leukaemia.

The sponsorship was transferred to Pfizer Limited, United Kingdom, in March 2013.

What is acute myeloid leukaemia?

Acute myeloid leukaemia is a disease in which cancer cells are found in the blood and the bone marrow. The bone marrow is the spongy tissue inside the large bones in the body. Normally, the bone marrow makes cells called “blasts” that mature into several different types of blood cells that have specific functions in the body. These include red cells, white cells and platelets. Red blood cells carry oxygen and other materials to all tissues of the body. White blood cells fight infection. Platelets make the blood clot. When leukaemia develops, the bone marrow produces large numbers of abnormal blood cells. There are several types of leukaemias. In myeloid leukaemia blasts that are developing into white blood cells called granulocytes are affected. These blasts do not mature but multiply abnormally and accumulate in the bone marrow. Then, they are also found in the blood. Leukaemia can be acute (when it develops quickly with many blasts). Acute myeloid leukaemia is life-threatening.

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

At the time of designation, acute myeloid leukaemia affected not more than 0.66 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 25,000 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 leukaemia is complex and depends on a number of factors including the type of leukaemia, the extent of the disease and whether the leukaemia has been treated before. It also depends on the age, the symptoms, and the general health of the patient. The primary treatment of acute myeloid leukemia is chemotherapy (using drugs to kill cancer cells). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation. Gemtuzumab ozogamicin, also known as mylotarg, could be of potential significant benefit for the treatment of acute myeloid leukaemia. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Antibodies are proteins in the body that target and link specific structures on the surface of foreign bodies, such as bacteria or cancer cells. CD33 is a protein found on the surface of acute myeloid leukaemia cells. Gemtuzumab ozogamicin is an antibody chemically linked to a specific compound that recognises and binds specifically to the CD33 protein. Following the binding, the cancer cells itself transforms the compound linked to the antibody into a type of toxic substance, which might lead to the destruction of the cancer cell.

What is the stage of development of this medicine?

The effects of gemtuzumab ozogamicin were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in patients with acute myeloid leukaemia were ongoing.

Gemtuzumab ozogamicin was marketed in the United States for the treatment of patients with CD33 positive acute myeloid leukaemia in first relapse who are 60 years of age or older and who are not considered candidates or cytotoxic chemotherapy, at the time of submission.

Orphan designation of Gemtuzumab ozogamicin was granted in the United States for the same indication and in Japan for patients with relapsed or refractory acute myelogenous (myeloid) leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 September 2000 recommending the granting of this designation.

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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.
At the time of designation, this represented a population of 375,500,000 (Eurostat 2000).

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[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Gemtuzumab ozogamicin	Treatment of acute myeloid leukaemia
Bulgarian	Гемтузумаб озогамицин	Лечение на остра миелоидна левкемия
Czech	Gemtuzumab ozagamicinu	Léčba akutní myeloidní leukémie
Danish	Gemtuzumab ozogamicin	Behandling af akut myeloid leukæmi
Dutch	Gemtuzumab-ozogamicine	Behandeling van acute myeloïde leukemie
Estonian	Gemtuzumabi ozogamitsiin	Akuutse müeloidse leukeemia ravi
Finnish	Gemtutsumabi otsogamisiini	Akuutin myelooisen leukemian hoito
French	Gemtuzumab ozogamicin	Traitement de la leucémie aiguë myéloïde
German	Gemtuzumab ozogamicin	Behandlung der akuten myeloischen Leukämie
Greek	Gemtuzumab ozogamicin	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Gemtuzumab ozogamicin	Akut myeloid leukaemia kezelése
Italian	Gemtuzumab ozogamicin	Trattamento della leucemia mieloide acuta
Latvian	Gemtuzumaba ozogamicīns	Akūtas mieloleikozes ārstēšana
Lithuanian	Gemtuzumabo ozogamicinas	Ūmios mieloleukozės gydymas
Maltese	Gemtuzumab ozogamicin	Kura tal-lewkimja mjelojda akuta
Polish	Gemtuzumab ozogamycyna	Leczenie ostrej białaczki szpikowej
Portuguese	Ozogamicina gentuzumabe	Tratamento da leucemia mielóide aguda
Romanian	Gemtuzumab ozogamicin	Tratamentul leucemiei mieloide acute
Slovak	Ozogamicíngemtuzumab	Liečba akútnej myeloidnej leukémie
Slovenian	Gemtuzumab ozogamicin	Zdravljenje akutne mieloične levkemije
Spanish	Gemtuzumab ozogamicina	Tratamiento de la leucemia mieloide aguda
Swedish	Gemtuzumab ozogamicin	Behandling av akut myeloisk leukemi

[1] At the time of transfer of sponsorship