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Public summary of opinion on orphan designation

Cusatuzumab for the treatment of acute myeloid leukaemia

On 22 April 2020, orphan designation EU/3/20/2265 was granted by the European Commission to Janssen-Cilag International N.V., Belgium, for cusatuzumab 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 infection). In patients with AML, the bone marrow (the spongy tissue inside the large bones where blood cells are produced) produces white blood cells that are not fully developed and do not work properly. These abnormal cells quickly build up in large numbers in the bone marrow and are then found in the blood.

AML is a life-threatening disease because these abnormal white blood cells replace the normal blood cells in the bone marrow and the blood, causing bleeding episodes, blood clots and reduced ability to fight infections.

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

At the time of designation, AML affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 83,000 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, symptoms and general state of health. At the time of designation, several medicines were authorised for treating AML. Patients might also receive stem cell transplantation, a

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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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.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with AML because early results from studies show that when given in combination with azacitidine (another cancer medicine), cusatuzumab could improve the response to treatment in patients who were not well enough to receive intensive chemotherapy (high doses of medicines that kill cancer cells or stop them growing). 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?

Cusatuzumab is a monoclonal antibody (a type of protein) designed to attach to a protein called CD70. This protein is found on cancer cells, including AML cells, and is involved in the growth of the cancer. By binding to CD70, cusatuzumab is expected to block the growth of the cancer, thus slowing down the progression of the disease.

What is the stage of development of this medicine?

The effects of cusatuzumab have been evaluated in experimental models.

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

At the time of submission, cusatuzumab was not authorised anywhere in the EU for the treatment of AML. Orphan designation of cusatuzumab had been granted in the USA for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 19 March 2020, 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.

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

[1] At the time of designation