5 January 2016
EMA/COMP/696064/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47 for the treatment of acute myeloid leukaemia

On 11 November 2015, orphan designation (EU/3/15/1582) was granted by the European Commission to The Chancellor, Masters and Scholars of the University of Oxford, United Kingdom, for humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47 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 less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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)

[*]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 512,900,000 (Eurostat 2015).

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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).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with AML because early studies in experimental models show that it might reduce the number of leukaemia cells and improve survival. 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?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a protein called CD47. CD47 is found on the surface of many cells and protects them against attacks by the body's immune system. CD47 is found at high levels on the surface of cancer cells. By attaching to CD47, the antibody is expected to block signals that stop certain immune cells from attacking cancer cells, thus subsequently contributing to the destruction of the cancer cells.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with AML had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for AML. Orphan designation of the medicine 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 8 October 2015 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	Humanised monoclonal antibody of the IgG4 kappa isotype targeting CD47	Treatment of acute myeloid leukaemia
Bulgarian	Хуманизирано моноклонално антитяло от IgG4 каппа изотип, насочено срещу CD47	Лечение на остра миелоидна левкемия
Croatian	Humanizirano monoklonsko protutijelo IgG4 izotipa kapa usmjereno na CD47	Liječenje akutne mijeloične leukemije
Czech	Humanizovaná monoklonální protilátka izotypu kappa IgG4, zaměřená proti CD47	Léčba akutní myeloidní leukémie
Danish	Humaniseret, monoklonalt antistof fra IgG4-kappa-isotypen rettet mod CD47	Behandling af akut myeloid leukæmi
Dutch	Gehumaniseerd monoklonaal antilichaam van het isotype IgG4 kappa, gericht tegen CD47	Behandeling van acute myeloïde leukemie
Estonian	Humaniseeritud monoklonaalne IgG4 kappa isotüüpi antikeha suunatud CD47-le.	Akuutse müeloidse leukeemia ravi
Finnish	CD47:ään kohdentuva IgG4:än kappa-isotyypin humanisoitu monoklonaalinen vasta-aine	Akuutin myelooisen leukemian hoito
French	Anticorps monoclonal humanisé d'isotype IgG4/ kappa dirigé contre le CD47	Traitement de la leucémie aiguë myéloïde
German	Humanisierter monoklonaler Antikörper des Isotyps IG64 kappa, gegen CD47 gerichtet	Behandlung der akuten myeloischen Leukämie
Greek	Ανθρωποποιημένο μονοκλωνικό αντίσωμα του ισότυπου ανοσοσφαιρίνης IgG4-κ που στοχεύει την πρωτεΐνη CD47	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	CD47-et célzó IgG4 kappa izotípus humanizált monoklonális antitestje	Akut myeloid leukaemia kezelése
Italian	Anticorpo monoclonale umanizzato dell'isotipo IgG4 kappa che ha come bersaglio la CD47	Trattamento della leucemia mieloide acuta
Latvian	Humanizēta IgG4 kappa izotipa mono-klonējuma antiviela, kas mērķē uz CD47	Akūtas mieloleikozes ārstēšana
Lithuanian	IgG4 kappa izotipo humanizuotas monokloninis antikūnas, veikiantis CD47	Ūmios mieloleukozės gydymas
Maltese	Antikorp monoklonali umanizzat tal-isotip IgG4 kappa mmirat lejn CD47	Kura tal-lewkimja mjelojda akuta
Polish	Humanizowane przeciwciało monoklonalne izotopu IgG4 kappa skierowane przeciw CD47	Leczenie ostrej białaczki szpikowej
Portuguese	Anticorpo monoclonal humanizado do isótipo IgG4 tipo kappa dirigido ao CD47	Tratamento da leucemia mielóide aguda
Romanian	Anticorp monoclonal umanizat al izotipului kappa IgG4 anti-CD47	Tratamentul leucemiei mieloide acute

[1] At the time of designation

Language	Active ingredient	Indication
Slovak	Humanizovaná monoklonálna protilátka IgG4 kappa izotypu, zacielená na CD47	Liečba akútnej myeloickej leukémie
Slovenian	Humanizirano monoklonsko protitelo izotipa IgG4 kappa, usmerjeno proti (proteinu) CD47	Zdravljenje akutne mieloične levkemije
Spanish	Anticuerpo monoclonal humanizado del isotipo IgG4 kappa contra CD47	Tratamiento de la leucemia mieloide aguda
Swedish	Humaniserad monoklonal antikropp av IgG4 kappa-isotypen mot CD47	Behandling av akut myeloisk leukemi
Norwegian	Menneskliggjort monoklonisk antilegeme av IgG4 kappa isotype med CD47 som målsetting	Behandling av akutt myelogen leukemi
Icelandic	Einstofna mannaaðlagað mótefni af IgG4 kappa samsætugerðinni sem miðar á CD47	Meðferð við bráðu kyrningahvítblæði