16 November 2016
EMA/622433/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Radio-iodinated (^{131}I) anti-CD45 murine monoclonal antibody for treatment in haematopoietic stem cell transplantation

On 14 October 2016, orphan designation (EU/3/16/1760) was granted by the European Commission to Wainwright Associates Ltd, United Kingdom, for radio-iodinated (^{131}I) anti-CD45 murine monoclonal antibody (also known as Iomab-B) for treatment in haematopoietic stem cell transplantation.

In November 2016, Wainwright Associates Ltd changed name to PharmaLex UK Services Limited.

What is haematopoietic stem cell transplantation?

Haematopoietic stem cell transplantation involves a patient receiving stem cells (cells that can develop into different types of cell) to help the bone marrow produce healthy blood cells. It can be used to treat serious diseases of the blood and immune system such as leukaemia. Before receiving the transplant, the patient's own bone marrow is cleared of cells. The patient then receives stem cells, which multiply and develop into healthy blood cells.

Haematopoietic stem cell transplantation can be a debilitating and life-threatening procedure due to the risk of severe infections and developing graft-versus-host disease (when the transplanted cells recognise the patient's body as 'foreign' and attack the patient's organs leading to organ damage).

What is the estimated number of patients receiving haematopoietic stem cell transplants?

At the time of designation, approximately 1 in 10,000 people in the European Union (EU) receive haematopoietic stem cell transplants per year. This was equivalent to a total of around 51,000 people per year*, and is below the ceiling for orphan designation. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

*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 (European Union (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

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What treatments are available?

At the time of designation, several treatments were available in the EU for patients undergoing haematopoietic stem cell transplants. These included radiation treatment or intensive treatment with cancer medicines such as busulfan to clear the bone marrow of existing cells, medicines to help restore the immune system, such as immunoglobulin replacement therapy, and medicines to reduce the risk of infections, such as antiviral and antifungal medicines.

The sponsor has provided sufficient information to show that radio-iodinated (^{131}I) anti-CD45 murine monoclonal antibody might be of significant benefit for patients receiving haematopoietic stem cell transplantation. Preliminary studies in patients with leukaemia that had come back or not responded to treatment, and who were not suitable for intensive treatments to clear the bone marrow, suggested that adding this medicine to conventional treatments before a transplant might improve how long such patients live. 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 made of a monoclonal antibody (a type of protein) that has been designed to recognise and attach to a receptor called CD45 found only on the surface of white blood cells and on the stem cells in the bone marrow that make them. The antibody is attached to a molecule containing a form of iodine, ^{131}I, that emits low-level radiation. When the medicine is given, the antibody attaches to these cells in the bone marrow, so the radiation can kill them without affecting other tissues elsewhere in the body. This clears the bone marrow so that donor stem cells can be given as a transplant.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients requiring treatment in haematopoietic stem cell transplantation were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for treatment in haematopoietic stem cell transplantation 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 8 September 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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Radio-iodinated (^{131}I) anti-CD45 murine monoclonal antibody	Treatment in haematopoietic stem cell transplantation
Bulgarian	Радио-йодирано(^{131}I) мише моноклонално антитяло срещу CD45	Лечение при трансплантация на хемопоетични стволови клетки
Croatian	Mišje monoklonsko protutijelo anti-CD45 obilježeno radioaktivnim jodom (^{131}I)	Liječenje u transplantaciji hematopoetskih matičnih stanica
Czech	Myší monoklonální protilátka proti CD45 značená radiojodem (^{131}I)	Léčba transplantace hemopoetickými zárodečnými buňkami
Danish	Radioaktivt joderet (^{131}I) anti-CD45 murint monoklonalt antistof	Behandling i hæmatopoietisk stamcelletransplantation
Dutch	Radiogejodeerd (^{131}I) anti-CD45 murien monoklonaal antilichaam	Behandeling in haematopoiëtische stemceltransplantatie
Estonian	Radiojodeeritud (^{131}I) CD45-vastane hiire monoklonaalne antikeha	Kasutamiseks hematopoeetiliste tüvirakkude transplantatsiooni ravis.
Finnish	Radiojodioitu (^{131}I) hiiren monoklonaalinen anti-CD45-vasta-aine	Hoito hematopoeettisen kantasolusiirron yhteydessä
French	Anticorps monoclonal murin anti-CD45 conjugué à de l'iode radioactif (^{131}I)	Traitement dans la greffe de moëlle osseuse
German	Radioiodierter (^{131}I) muriner monoklonaler Anti-CD45-Antikörper	Behandlung in hämatopoetischer Stammzelltransplantation
Greek	Ραδιο-ιωδιωμένο (^{131}I) αντι-CD45 μονοκλωνικό αντίσωμα ποντικού	θεραπεία σε μεταμόσχευση αρχέγονων αιμοποιητικών κυττάρων
Hungarian	Radiojódozott (^{131}I) anti-CD45 murin monoklonális antitest	Hematopoietikus őssejt-transzplantáció esetén alkalmazandó
Italian	Anticorpo monoclonale murino radio iodato (^{131}I) anti-CD45	Trattamento nel trapianto di cellule staminali ematopoietiche
Latvian	Radioaktīvo jodu (^{131}I) saturoša peles monoklonālā anti-CD45 antiviela	Ārstēšanai hematopoētisko cilmes šūnu transplantācijā
Lithuanian	Radioaktyviuoju jodu žymėtas (^{131}I) pelės monokloninis antikūnas prieš CD45	Taikoma hematopoetinių kamieninių ląstelių transplantacijų gydyme
Maltese	Antikorp monoklonali tal-ġrieden kontra CD45 b'jodju radjuattiv (^{131}I)	Kura fi trapjant ta' ċelloli staminali ematopojetiċi
Polish	Znakowane radioaktywnym jodem (^{131}I) mysie przeciwciało monoklonalne anty-CD45	Leczenie w przebiegu przeszczepu hematopoetycznych komórek macierzystych
Portuguese	Anticorpo monoclonal de murino anti-CD45 radioiodado (^{131}I)	Tratamento em transplantes de células estaminais hematopoiéticas
Romanian	Anticorp monoclonal anti-CD45 murin radioiodat (^{131}I)	Tratament în transplantul de celule stem hematopoetice
Slovak	Myšia monoklonálna protilátka proti CD45 značená rádiojódom (^{131}I)	Liečba pri transplantácii hematopoietických kmeňových buniek

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Mišje monoklonsko protitelo proti CD45, označeno z radioaktivnim jodom (^{131}I)	Zdravljenje pritransplantaciji hematopoetskih matičnih celic
Spanish	Anticuerpo monoclonal murino anti-CD45 radiomarcado con yodo (^{131}I)	Tratamiento en el trasplante de células madre hematopoyéticas
Swedish	Radiojoderad (^{131}I) anti-CD45 murin monoklonal antikropp	Behandling vid hematopoetisk stamcellstransplantation
Norwegian	Murint monoklonalt antistoff mot CD45 merket med radioaktivt jod (^{131}I)	Behandling ved hematopoetisk stamcelletransplantasjon
Icelandic	Einstofna geislajoðs (^{131}I) músamótefni gegn CD45	Meðferð á stofnfrumublóðfrumu ígræðslu