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Public summary of opinion on orphan designation

Recombinant human monoclonal antibody against mannan-binding lectin-associated serine protease-2 for the treatment in haematopoietic stem cell transplantation

On 24 August 2018, orphan designation (EU/3/18/2067) was granted by the European Commission to Omeros London Limited, United Kingdom, for recombinant human monoclonal antibody against mannan-binding lectin-associated serine protease-2 (also known as OMS721) for treatment in haematopoietic stem cell transplantation.

What is haematopoietic stem cell transplantation?

Haematopoietic stem cell transplantation (HSCT) is a procedure where the patient's bone marrow is cleared of cells and replaced by stem cells (cells that can develop into different types of cell) from a donor to form new bone marrow that produces healthy blood cells. It can be used to treat serious diseases of the blood and immune system such as leukaemia.

HSCT 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 regard the patient's body as 'foreign' and attack the patient's organs, leading to organ damage). It can also lead to a condition known as thrombotic microangiopathy, where blood clots occur in small blood vessels.

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

At the time of designation, approximately 1 in 10,000 people in the European Union (EU) receive HSCT per year in the European Union (EU). This was equivalent to a total of around 52,000 people*, 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 (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).

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What treatments are available?

At the time of designation, several medicines were authorised in the EU for patients undergoing HSCT. 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 filgrastim, immunoglobulin replacement therapy and Zalmoxis, and medicines to reduce the risk of infections, such as antiviral and antifungal medicines. Medicines that suppress the immune system, such as ciclosporin and corticosteroids, were used for the treatment of graft-versus-host disease. However, there were no products authorised for thrombotic microangiopathy.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients undergoing HSCT. Studies showed improved survival compared with other existing treatments when this medicine was used to treat patients with thrombotic microangiopathy after HSCT.

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?

The medicine is a monoclonal antibody designed to attach to a protein (MASP-2) involved in the formation of blood clots seen in thrombotic microangiopathy. Attaching to and blocking the action of this protein is expected to prevent blood clots in small blood vessels and thereby improve outcomes in patients undergoing HSCT.

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, clinical trials with the medicine in patients undergoing HSCT were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for in patients undergoing HSCT. Orphan designation of this medicine had been granted in the United States for complement-mediated thrombotic microangiopathy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 July 2018 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	Recombinant human monoclonal antibody against mannan-binding lectin-associated serine protease-2	Treatment in haematopoietic stem cell transplantation
Bulgarian	Рекомбинантно моноклонално антитяло срещу манан-свързваща, лектин-асоциирана серин протеаза-2	Лечение при трансплантация на хемопоетични стволови клетки
Croatian	Rekombinantno ljudsko monoklonsko protutijelo protiv manan-vezajuće serin proteaze 2 povezane s lektinom	Liječenje u transplantaciji hematopoetskih matičnih stanica
Czech	Rekombinantní humánní monoklonální protilátka proti mannan-vázajícícho lektinu asociovaného se serinovou proteásou-2	Léčba transplantace hemopoetickými zárodečnými buňkami
Danish	Rekombinant humant monoklonalt antistof mod mannan-bindende lektin-associeret serinprotease-2	Behandling i hæmatopoietisk stamcelletransplantation
Dutch	Recombinant humaan monoclonaal antilichaam gericht tegen mannan-bindend lectine-geassocieerd serine protease-2	Behandeling in haematopoiëtische stemceltransplantatie
Estonian	Mannaani -siduva lektiiniga seotud seriini proteaas 2 vastane rekombinantne inimese monoklonaalne antikeha	Kasutamiseks hematopoeetiliste tüvirakkude transplantatsiooni ravis
Finnish	Rekombinantti ihmisen monoklonaalinen vasta-aine mannaaniin sitoutuvaa, lektiiniin assosioutuvaa proteaasi 2:ta vastaan.	Hoito hematopoeettisen kantasolusiirron yhteydessä
French	Anticorps monoclonal humain recombinant dirigé contre la sérine protéase-2 associée à la lectine liant le mannose	Traitement dans la greffe de moëlle osseuse
German	Rekombinanter humaner monoklonaler Antikörper gegen Mannan-Binding Lectin-Associated Serine Protease 2	Behandlung in hämatopoetischer Stammzelltransplantation
Greek	Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα έναντι της προσδένουσας σε μαννάνη σχετιζόμενης με λεκτίνη πρωτεάσης σερίνης-2	θεραπεία σε μεταμόσχευση αρχέγονων αιμοποιητικών κυττάρων
Hungarian	Mannan-kötő lektin-asszociált szerin proteáz-2 elleni rekombináns humán monoklonális antitest	Hematopoietikus őssejt-transzplantáció esetén alkalmazandó
Italian	Anticorpo monoclonale umano ricombinante contro la serina proteasi-2 associata a la lectina che lega il mannoso	Trattamento nel trapianto di cellule staminali ematopoietiche
Latvian	Rekombinanta cilvēka monoklonālā antiviela, kas vērsta pret mannān-saistošu lecitīn-asociētu serīna proteāzi-2	Ārstēšanai hematopoētisko cilmes šūnu transplantācijā

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnas prieš mananą surišančią su lektinu asocijuotą serino proteazę-2	Taikoma hematopoetinių kamieninių ląstelių transplantacijų gydyme
Maltese	Antikorp monoklonali uman rikombinanti kontra proteaži-2 serina assoċjata ma' lektina u li jeħel ma' mannan	Kura fi trapjant ta' ċelloli staminali ematopojetiċi
Polish	Recombinowane ludzkie przeciwcialo monoklonalne przeciwko proteazie serynowej-2 wiążącej mannan i skojarzonej z lektyną	Leczenie w przebiegu przeszczepu hematopoetycznych komórek macierzystych
Portuguese	Anticorpo monoclonal humano recombinante contra a serina protease-2 associada à lectina de ligação à manose	Tratamento em transplantes de células estaminais hematopoiéticas
Romanian	Anticorp uman monoclonal recombinant împotriva serin proteazei-2 asociate cu MBL ("mannan-binding lectin")	Tratament în transplantul de celule stem hematopoetice
Slovak	Rekombinantná ľudská monoklonálna protilátka proti mannan-viažucej lektín-asociovanej serín proteáze-2	Liečba pri transplantácii hematopoietických kmeňových buniek
Slovenian	Recombinantno humano monoklonsko protitelo proti manozo vezajoči, z lektinom povezani serinski proteazi-2	Zdravljenje pritransplantaciji hematopoetskih matičnih celic
Spanish	Anticuerpo humano monoclonal recombinante contra la protease 2 serina asociada a la lectina liada a manan.	Tratamiento en el trasplante de células madre hematopoyéticas
Swedish	Rekombinant human monoklonal antikropp mot mannan-bindande lektin-associerat serinproteas-2	Behandling vid hematopoetisk stamcellstransplantation
Norwegian	Rekombinant humant monoklonalt antistoff mot mannanbindende lektinassosiert serinprotease-2	Behandling ved hematopoetisk stamcelletransplantasjon
Icelandic	Raðbrigða manna einstofna mótefni gegn mannan- bindandi lektín-tengdum serín próteasa-2	Meðferð á stofnfrumublóðfrumu ígræðslu