22 November 2018
EMA/533264/2018

Public summary of opinion on orphan designation

CD34+ haematopoietic stem and progenitor cells with CD3+ T-cells for treatment in solid organ transplantation

On 24 August 2018, orphan designation (EU/3/18/2063) was granted by the European Commission to IQVIA RDS Ireland Limited, Ireland, for CD34+ haematopoietic stem and progenitor cells with CD3+ T-cells for treatment in solid organ transplantation.

What is solid organ transplantation?

Solid organ transplantation is a surgical procedure in which a diseased organ, such as the heart, lungs, liver or kidney, is replaced with an organ from a donor.

Transplantation is a very complex procedure. During transplantation, the organ to be transplanted can become damaged because of the interruption and restoration of blood supply to the organ. In addition, graft rejection can occur after transplantation, when the recipient's body rejects the transplanted organ. Graft rejection is caused by the patient's immune system (the body's natural defences) recognising the transplanted graft as 'foreign' and attacking it.

These complications can be debilitating and life-threatening because they may result in the transplanted organ not working properly.

What is the estimated number of patients receiving solid organ transplants?

At the time of designation, approximately 0.7 in 10,000 people in the European Union (EU) were undergoing solid organ transplantation every year. This was equivalent to a total of 36,000 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).

What treatments are available?

At the time of designation, several medicines were authorised in the EU to prevent or treat graft rejection in solid organ transplantation. These include antibodies such as antilymphocyte

*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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immunoglobulin and thymoglobulin and other medicines that suppress immune processes such as azathioprine, basilixmab, ciclosporin, mycophenolate mofetil, tacrolimus, and corticosteroids such as prednisolone or methylprednisolone.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients undergoing solid organ transplantation. Early studies indicate that the medicine could help the recipient's body accept the transplant, allowing the patient to reduce use of medicines that suppress the immune system.

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?

To make the medicine, stem cells (cells that can develop into different types of blood cells) and T cells (cells of the immune system) are collected from the organ donor. These cells are processed and then given to the patient who has received the transplant, where they are expected to grow and multiply. This results in the patient's immune system containing a mixture of their own immune cells and the donor's immune cells. This is expected to prevent the immune system recognising the transplanted donor organ as 'foreign' and attacking it, and increase the chances that the transplant is successful.

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 solid organ transplantation were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for solid organ 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 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	CD34+ haematopoietic stem and progenitor cells with CD3+ T-cells	Treatment in solid organ transplantation
Bulgarian	CD34+ хематопоетични стволови и прогениторни клетки с CD3+ Т-клетки	Лечение при трансплантация на солиден орган
Croatian	CD34+ hematopoetske matične stanice i progenitorske stanice s CD3+ T stanicama	Liječenje u transplantaciji solidnih organa
Czech	CD34+ hematopoetické kmenové a progenitorové buňky s CD3+ T buňkami	Léčba pro transplantaci solidních orgánů
Danish	CD34+ hæmatopoietiske stam- og progenitorceller med CD3+ T-celler	Behandling i organ transplantation
Dutch	CD34+ hematopoëtische stam- en voorlopercellen met CD3+ T-cellen	Behandeling bij soliede orgaantransplantatie
Estonian	CD34+ hematopoeetilised tüvi- ja eellasrakud koos CD3+ T-rakkudega	Kasutamiseks elundite siirdamise ravis
Finnish	CD34+ hematopoieettiset kanta- ja progenitorisolut yhdessä CD3+ -T-solujen kanssa	Hoito kiinteän elimen siirron yhteydessä
French	CD34+ cellules souches et progénitrices hématopoïétiques avec cellules T CD3+	Traitement pour la transplantation d'organes solides
German	CD34+ hämatopoetische Stamm- und Vorläuferzellen mit CD3+ T-Zellen	Behandlung in Organtransplantation
Greek	CD34+ αιματοποιητικά βλαστικά και προγονικά κύτταρα με CD3+ Τ κύτταρα	Θεραπεία στη μεταμόσχευση συμπαγών οργάνων
Hungarian	CD34+ hematopoietikus ős- és progenitor sejtek CD3+ T sejtekkel	Szervtranszplantáció esetén alkalmazandó
Italian	Staminali ematopoietiche CD34+ e cellule progenitrici con cellule T CD3+	Trattamento nel trapianto di organi solidi
Latvian	CD34+ hematopoētiskās cilmes un progenitoru šūnas ar CD3+ T šūnām	Ārstēšanai norobežoto orgānu transplantācijā
Lithuanian	CD34+ hematopoetinės kamieninės ir ląstelių pirmtakai su CD3+ T ląstelėmis	Transplantacijos parenchiminiame organe gydymas
Maltese	CD34+ ċelloli staminali u proġenituri ematopojetiċi b'ċelluli T CD3+	Kura fi trapjant ta' organi solidi
Polish	Komórki macierzyste i progenitorowe CD34+ z limfocytami T CD3+	Leczenie w przebiegu transplantacji organów
Portuguese	Células estaminais e progenitoras hematopoiéticas CD34+ com células T CD3+	Tratamento em transplante de órgãos sólidos
Romanian	Celule stem și celule progenitoare hematopoietice CD34+ cu celule T CD3+	Tratament în transplantul de organe solide
Slovak	CD34+ hematopoetické kmeňové a progenitorové bunky s CD3+ T bunkami	Liečba pri transplantácii celého orgánu

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	CD34+ hematopoetske matične in progenitorske celice s CD3+ T celicami	Zdravljenje pri transplantaciji organov
Spanish	CD34+ células madre y progenitoras hematopoyéticas con células T CD3+	Tratamiento del trasplante de órgano sólido
Swedish	CD34+ hematopoetisk stam- och progenitorceller med CD3+ T-celler	Behandling vid organtransplantation.
Norwegian	CD34+ hematopoietiske stam- og forløperceller med CD3+ T-celler	Behandling ved transplantasjon av solide organer
Icelandic	CD34+ blóðmyndandi stofnfrumur með CD3+ T frumum	Meðferð við líffæraígræðslu