



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded for the treatment of graft-versus-host disease

On 31 July 2018, orphan designation (EU/3/18/2044) was granted by the European Commission to medac Gesellschaft für klinische Spezialpräparate mbH, Germany, for allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded for the treatment of graft-versus-host disease.

What is graft-versus-host disease?

Graft-versus-host disease is a complication that can occur in patients who have had a transplant (graft). In this disease, the transplanted cells recognise the patient's body as 'foreign' and attack the patient's organs, such as the stomach, gut, skin and liver, leading to organ damage. The disease may occur shortly after transplantation or later on, in which case a wider range of organs can be involved.

Graft-versus-host disease is a serious and life-threatening disease with a high mortality rate.

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

At the time of designation, graft-versus-host disease affected approximately 0.16 in 10,000 people in the European Union (EU). This was equivalent to a total of around 8,000 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?

At the time of designation, several medicines were authorised in the European Union (EU) for the treatment of graft-versus-host disease, such as ciclosporin and corticosteroids. Treatment aimed at reducing the activity of transplanted cells involved in graft-versus-host disease, thereby reducing their ability to attack the patient's organs.

¹ Correction of translation table

² Translations updated following corrigendum of 31 July 2021.

*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).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with graft-versus-host disease because early data showed that the medicine was effective in patients in whom corticosteroids and ciclosporin did not work.

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 contains human mesenchymal stromal cells (MSCs) from donors which are then grown in a laboratory to increase their numbers. In the body, MSCs help to regulate the immune system, reducing the activation and growth of cells responsible for attacking foreign bodies. The medicine, which is to be given by injection, is expected to reduce immune activity, decreasing inflammation and damage to the affected organs.

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, studies with the medicine in patients with graft-versus-host disease were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for graft-versus-host disease 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 21 June 2018 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 languages³, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Allogeneic bone marrow derived mesenchymal stromal cells, ex-vivo expanded	Treatment of graft-versus-host disease
Bulgarian	Алогенни мезенхимни стромални клетки, извлечени от костен мозък, разширени ex-vivo	Лечение на болестта на присадката срещу приемателя
Croatian	Alogenične mezenhimalne stromalne stanice iz koštane srži, ekspandirane ex vivo	Liječenje reakcije presatka protiv primatelja
Czech	Alogenní mezenchymální stromální buňky odvozené z kostní dřevě, expandované ex vivo	Léčba reakce štěpu proti hostiteli
Danish	Allogene knoglemarvsaffedte mesenkymale stromaceller, ekspanderet ex vivo	Behandling af graft versus host reaktion
Dutch	Uit allogeen beenmerg afgeleide mesenchymale stromacellen, ex-vivo geëxpandeerd	Behandeling van graft versus host ziekte
Estonian	Allogeensed luuüdist pärinevad mesenhümaalsed stroomarakud, ex vivo laiendatud	Graft versus host haiguse ravi
Finnish	Allogeenisesta luuytimeistä peräisin olevat ex-vivo-viljellyt mesenkymaaliset stroomasolut	Käänteishyljintäreaktion hoito
French	Cellules stromales mésenchymateuses allogéniques dérivées de moelle osseuse, développées ex vivo	Traitement de la réaction du greffon contre l'hôte
German	Allogene, aus Knochenmark isolierte mesenchymale Stromazellen, ex vivo expandiert	Behandlung der Graft-versus-Host-Reaktion
Greek	Αλλογενή μεσεγχυματικά στρωματικά κύτταρα μυελού των οστών, πολλαπλασιασμένα εργαστηριακά (ex-vivo)	Θεραπεία της αντίδρασης του μοσχεύματος
Hungarian	Allogén csontvelő-eredetű mesenchymalis sztrómasejtek, ex vivo expandált	Graft-versus-host betegség kezelése
Italian	Cellule stromali mesenchimali derivate da midollo osseo allogenico, espanse ex vivo	Trattamento della reazione del trapianto contro l'ospite
Latvian	Alogēnas, no kaulu smadzenēm atvasinātas mezenhimālas stromas šūnas, kas paplašinātas ex-vivo	Saimnieka-transplantāta slimības ārstēšana
Lithuanian	Ex vivo pagausintos iš alogeninių kaulų čiulpų išskirtos mezenchiminės stromos ląstelės	Transplantato atmetimo ligos gydymas
Maltese	Ċelloli stromali meżenkimali li ġejjin mill-mudullun alloġeniku, espanduti ex vivo	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Mezenchymalne komórki zrębu ze szpiku kostnego dawcy allogenicznego, ekspandowane w warunkach ex vivo	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Células estromais mesenquimais alogénicas, derivadas da medula óssea, expandidas ex-vivo	Tratamento da reacção do enxerto contra o hospedeiro
Romanian	Celule stromale mezenchimale alogene derivate din măduvă osoasă, expandate ex-vivo	Tratamentul reacției grefei contra gazdei

³ At the time of designation

Language	Active ingredient	Indication
Slovak	Alogénne mezenchymálne stromálne bunky odvodené z kostnej drene expandované ex vivo	Liečba reakcie štepú proti hostiteľovi
Slovenian	Alogenske mezenhimske stromalne celice, pridobljene iz kostnega mozga, razširjene <i>ex vivo</i>	Zdravljenje bolezni presadka proti gostitelju
Spanish	Células estromales mesenquimales alogénicas derivadas de médula ósea, propagadas <i>ex vivo</i>	Tratamiento de la enfermedad de injerto contra huésped
Swedish	Allogena mesenkymala stromaceller som härrör från benmärg, expanderade ex vivo	Behandling av graft-värd host reaktion