



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.

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.

^{*}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).



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 languages¹, 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	Възрастни човешки алогенни мезенхимни стромални клетки, култивирани ex-vivo	Лечение на болестта на присадката срещу приемателя
Croatian	Ex-vivo umnožene odrasle ljudske alogene mezenhimalne stromalne stanice	Liječenje reakcije presatka protiv primatelja
Czech	Ex-vivo expandované dospělé humánní alogenní mesenchymální stromální buňky	Léčba reakce štěpu proti hostiteli
Danish	Voksne humane allogene mesenkymale stromale celler, ekspanderede ex vivo	Behandling af graft versus host reaktion
Dutch	Ex-vivo geëxpandeerde adult humane allogene mesenchymale stromale cellen	Behandeling van graft versus host ziekte
Estonian	Kehaväliselt paljundatud täiskasvanud inimese allogeensed mesenhümaalsed stroomarakud	Graft versus host haiguse ravi
Finnish	Aikuisen ihmisen ex-vivo laajennetut allogeeniset, mesenkymaaliset stroomasolut	Käänteishyljintäreaktion hoito
French	Cellules stromales mésenchymateuses humaines adultes allogènes amplifiées ex vivo	Traitement de la réaction du greffon contre l'hôte
German	Ex vivo expandierte adulte humane allogene mesenchymale Stromazellen	Behandlung der Graft-versus-Host-Reaktion
Greek	Ex-vivo πολλαπλασιασμένα ανθρώπινα αλλογενή μεσεγγυματικά στρωματικά κύτταρα ενήλικου	Θεραπεία της αντίδρασης του μοσχεύματος
Hungarian	Ex vivo tenyésztett felnőtt humán allogén mesenchymalis stromasejtek	Graft-versus-host betegség kezelése
Italian	Cellule stromali mesenchimali allogeniche umane adulte espanse ex vivo	Trattamento della reazione del trapianto contro l'ospite
Latvian	Ex-vivo pavairotas pieauguša cilvēka alogēnās mezenhīmas stromas šūnas	Saimnieka-transplantāta slimības ārstēšana
Lithuanian	Ex-vivo pagausintos suaugusio žmogaus alogeninės mezenchimos stromos ląstelės	Transplantato atmetimo ligos gydymas
Maltese	Ċelloli stromali mesencimali alloġeniċi tal-bniedem adult espanduti ex-vivo	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Ludzkie allogeniczne mezenchymalne komórki zrębu dorosłych namnożone w warunkach ex vivo	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Células estromais mesenquimais humanas adultas alogénicas expandidas ex-vivo	Tratamento da reacção do enxerto contra o hospedeiro
Romanian	Celule stromale mezenchimale alogene umane adulte, expandate ex-vivo	Tratamentul reacției grefei contra gazdei
Slovak	Allogénne mezenchýmové stromálne bunky pochádzajúce z dospelého človeka, expandované ex vivo	Liečba reakcie štepu proti hostiteľovi

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Alogenske mezenhimske stromalne celice odraslih ljudi, ekspandirane ex vivo	Zdravljenje bolezni presadka proti gostitelju
Spanish	Células madre mesenquimales humanas adultas alogénicas expandidas ex-vivo	Tratamiento de la enfermedad de injerto contra huésped
Swedish	Ex vivo-expanderade humana allogena mesenkymala stromaceller från vuxna	Behandling av graft-värd host reaktion
Norwegian	Ex vivo-ekspanderte humane allogene mesenkymale stromale celler fra voksne	Behandling av graft-versus-host - reaksjon
Icelandic	Ósamgena bandvefsgrunnfrumur úr fullorðnum mönnum sem er fjölgað utan líkamans (ex-vivo)	Til meðferðar á hýsilssótt