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Public summary of opinion on orphan designation

Allogeneic faecal microbiota, pooled for the treatment of graft-versus-host disease

On 19 November 2018, orphan designation (EU/3/18/2083) was granted by the European Commission to MaaT Pharma, France, for allogeneic faecal microbiota, pooled 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. 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 more organs can be affected.

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 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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 EU for the treatment of graft-versus-host disease, such as ciclosporin and corticosteroids. Treatment aimed to reduce 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. Published studies showed that the medicine can

*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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reduce the effects of graft-versus-host disease when given together with standard treatment, and in patients who no longer respond to authorised treatments.

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 human gut hosts several types of bacteria and other microorganisms, which together are known as microbiota. The microbiota plays an important role not only in the digestive process, but also in maintaining the correct functioning of the immune system (the body's natural defences). A disruption of the balance within the microbiota, sometimes caused by medical treatment, can cause uncontrolled responses of the immune system and inflammation throughout the body.

This medicine is made of microbiota from healthy donors. Once it is given to a patient with graft-versus-host disease, the medicine is expected to help re-establish a balanced microbiota in the gut of the patient, reducing inflammation and regulating the immune system. This, in turn, is expected to help protect the patient from the effect of graft-versus-host disease.

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 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 11 October 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	Allogeneic faecal microbiota, pooled	Treatment of graft-versus-host disease
Bulgarian	Алогенна фекална микрофлора, смесена	Лечение на болестта на присадката срещу приемателя
Croatian	Alogenična fekalna mikrobiota, pulirana	Liječenje reakcije presatka protiv primatelja
Czech	Allogenní fekální mikrobiota, směsná	Léčba reakce štěpu proti hostiteli
Danish	Allogen fækal mikrobiota, samlet	Behandling af graft versus host reaktion
Dutch	Allogene fecale microbiota, Gepoolde	Behandeling van graft versus host ziekte
Estonian	Allogeenne fekaalne mikroobikooslus, puulitud	Graft versus host haiguse ravi
Finnish	Allogeeninen fekaalinen, poolattu mikrobisto	Käänteishyljintäreaktion hoito
French	Microbiote fécal allogénique, pool	Traitement de la réaction du greffon contre l'hôte
German	Allogene fäkale Mikrobiota, gepoolt	Behandlung der Graft-versus-Host-Reaktion
Greek	Αλλογενή ομαδοποιημένα μικροβιοτικά κοπράνων	Θεραπεία της αντίδρασης του μοσχεύματος
Hungarian	Allogén fecal microbiota, összesített	Graft-versus-host betegség kezelése
Italian	Pool di microbiota fecale allogenico	Trattamento della reazione del trapianto contro l'ospite
Latvian	Allogēnais fekālais mikrobiots, apvienots	Saimnieka-transplantāta slimības ārstēšana
Lithuanian	Alogeninis, atrinktas iš kelių šaltinių, išmatų mikrobiomas	Transplantato atmetimo ligos gydymas
Maltese	Mikrobijota fl-ippurgar alloġeniku, miġbura	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Próbka zbiorcza allogenicznej mikroflory kałowej	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Flora microbiana fecal alogénica, agrupada	Tratamento da reacção do enxerto contra o hospedeiro
Romanian	Microbiotă fecală alogenică, grupată	Tratamentul reacţiei grefei contra gazdei
Slovak	Alogenická fekálna mikroflóra, združená	Liečba reakcie štepu proti hostiteľovi
Slovenian	Alogenska fekalna mikrobiota, združena	Zdravljenje bolezni presadka proti gostitelju
Spanish	Microbiota fecal alogénica, agrupada	Tratamiento de la enfermedad de injerto contra huésped
Swedish	Allogen fekal microbiota, poolad	Behandling av graft-värd host reaktion
Norwegian	Allogen fekal microbiota, sammenslått fra flere donorer	Behandling av graft-versus-host -reaksjon
Icelandic	Ósamgena saurörverur, samsafnaðar	Til meðferðar á hýsilssótt

[1] At the time of designation