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EMA/COMP/505608/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cannabidiol for the treatment of graft-versus-host disease

On 29 August 2016, orphan designation (EU/3/16/1718) was granted by the European Commission to Richardson Associates Regulatory Affairs Ltd, United Kingdom, for cannabidiol for the treatment of graft-versus-host disease.

What is graft-versus-host disease?

Graft-versus-host disease is a complication that can affect patients who have had allogeneic haematopoietic (blood) stem-cell transplantation to treat diseases of the blood such as leukaemia (a cancer of the white blood cells). The procedure involves a patient receiving stem cells from a matched donor to help restore the bone marrow, which produces new blood cells.

Graft-versus-host disease occurs when 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 happen 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 less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 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 to

*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 513,700,000 (Eurostat 2016).

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 cannabidiol might be of significant benefit for patients with graft-versus-host disease because preliminary studies showed that patients who did not respond to other treatment responded to cannabidiol. 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?

Following stem-cell transplantation, damage caused by the procedure causes inflammation of the patient's organs and stimulates the transplanted cells to attack the body, resulting in graft-versus-host disease. Cannabidiol reduces inflammation and other damaging immune effects and it is thereby expected to protect the patient's organs from injury.

Cannabidiol is present in the cannabis plant but it is not expected to have effects on mood, mental activity or behaviour.

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, cannabidiol was not authorised anywhere in the EU for treatment of graft-versus-host disease. Orphan designation of the medicine had been granted in the EU and in the United States for the prevention of graft-versus-host disease.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2016 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	Cannabidiol	Treatment of graft-versus-host disease
Bulgarian	Канабидиол	Лечение на болестта на присадката срещу приемателя
Croatian	Kanabidiol	Liječenje reakcije presatka protiv primatelja
Czech	Kanabidiol	Léčba reakce štěpu proti hostiteli
Danish	Cannabidiol	Behandling af graft versus host reaktion
Dutch	Cannabidiol	Behandeling van graft versus host ziekte
Estonian	Kannabidiool	Graft versus host haiguse ravi
Finnish	Kannabidioli	Käänteishyljintäreaktion hoito
French	Cannabidiol	Traitement de la réaction du greffon contre l'hôte
German	Cannabidiol	Behandlung der Graft-versus-Host-Reaktion
Greek	κανναβιδιόλη	Θεραπεία της αντίδρασης του μοσχεύματος
Hungarian	Kannabidiol	Graft-versus-host betegség kezelése
Italian	Cannabidiolo	Trattamento della reazione del trapianto contro l'ospite
Latvian	Kanabidiols	Saimnieka-transplantāta slimības ārstēšana
Lithuanian	Kanabidiolis	Transplantato atmetimo ligos gydymas
Maltese	Cannabidiol	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Kannabidiol	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Canabidiol	Tratamento da reação do enxerto contra o hospedeiro
Romanian	Canabidiol	Tratamentul reacției grefei contra gazdei
Slovak	Kanabidiol	Liečba reakcie štepů proti hostiteľovi
Slovenian	Kanabidiol	Zdravljenje bolezni presadka proti gostitelju
Spanish	Cannabidiol	Tratamiento de la enfermedad de injerto contra huésped
Swedish	Cannabidiol	Behandling av graft-vård host reaktion
Norwegian	Kannabidiol	Behandling av graft-versus-host -reaksjon
Icelandic	Kannabídíól	Til meðferðar á hýsilssótt

¹ At the time of designation