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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cannabidiol for the prevention of graft-versus-host disease

On 28 April 2016, orphan designation (EU/3/16/1645) was granted by the European Commission to Richardson Associates Regulatory Affairs Ltd, UK, for cannabidiol for the prevention of graft-versus-host disease.

What is graft-versus-host disease?

Graft-versus-host disease is a complication that can affect patients who have received 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 the bone marrow produce healthy 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 it may be delayed, 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 at risk of developing the condition?

At the time of designation, the number of patients at risk of graft-versus-host disease was estimated to be approximately 0.3 people in 10,000 in the European Union (EU). This was equivalent to a total of around 15,000 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).

*Disclaimer: For the purpose of the designation, the number of patients at risk of developing 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).

What methods of prevention are available?

At the time of designation, several medicines were authorised in the EU for the prevention of graft-versus-host disease, such as ciclosporin and antilymphocyte immunoglobulins. Treatment aimed to reduce the activity of the transplanted immune cells, 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 at risk of graft-versus-host disease because preliminary studies suggest that the medicine can help prevent acute disease when added to other 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?

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 acts to reduce 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 cannabidiol have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with cannabidiol in patients at risk of graft-versus-host disease were ongoing.

At the time of submission, cannabidiol was not authorised anywhere in the EU for the prevention of graft-versus-host disease. Orphan designation of the medicine had been granted in the US 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 23 March 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	Prevention of graft-versus-host disease
Bulgarian	Канабидиол	Профилактика на болест на присадката срещу приемателя
Croatian	Kanabidiol	Prevenција reakcije presatka protiv primatelja
Czech	Kanabidiol	Prevence onemocnění štěpu proti hostiteli
Danish	Cannabidiol	Forebyggelse af graft versus host reaktion
Dutch	Cannabidiol	Preventie van "graft versus host" ziekte
Estonian	Kannabidiool	<i>Graft versus host</i> haiguse preventatsioon
Finnish	Kannabidioli	Käänteishyljintäreaktion esto
French	Cannabidiol	Prévention de la réaction du greffon contre l'hôte
German	Cannabidiol	Prävention der Graft-versus-Host-Reaktion
Greek	κανναβιδιόλη	Πρόληψη της αντίδρασης του μοσχεύματος
Hungarian	Kannabidiol	Graft-versus-host betegség megelőzése
Italian	Cannabidiolo	Prevenzione della reazione del trapianto contro l'ospite
Latvian	Kanabidiols	Saimnieka-transplantāta slimības novēršana
Lithuanian	Kanabidiolis	Transplantato atmetimo ligos prevencija
Maltese	Cannabidiol	Kura tal-marda tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Kannabidiol	Zapobieganie chorobie przeszczep przeciw gospodarzowi
Portuguese	Canabidiol	Prevenção da doença do enxerto contra o hospedeiro
Romanian	Canabidiol	Prevenirea bolii grefă contra gazdă
Slovak	Kanabidiol	Prevenia reakcie štepů proti hostiteľovi
Slovenian	Kanabidiol	Preprečevanje reakcije presadka proti gostitelju
Spanish	Cannabidiol	Prevención de la enfermedad de injerto contra huésped
Swedish	Cannabidiol	Förebyggande av graft-värd host reaktion
Norwegian	Kannabidiol	Forebygging av graft-versus-host -reaksjon
Icelandic	Kannabídíól	Forvörn gegn hýsilssótt

¹ At the time of designation