



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

8 March 2018  
EMA/5287/2018

## Public summary of opinion on orphan designation

### Cannabidiol for the treatment of tuberous sclerosis

On 17 January 2018, orphan designation (EU/3/17/1959) was granted by the European Commission to GW Research Ltd, United Kingdom, for cannabidiol for the treatment of tuberous sclerosis.

#### What is tuberous sclerosis?

Tuberous sclerosis is a genetic disease that causes growth of benign (non-cancerous) tumours in different organs of the body, including the brain, lungs, heart, kidneys, skin and eyes. The symptoms and severity of the disease vary from patient to patient. Depending on where the tumours are, symptoms may include epilepsy, learning difficulties, skin abnormalities and kidney problems.

Tuberous sclerosis is a long-term debilitating disease that can be life threatening in patients with severe symptoms, who may develop severe learning disability, uncontrollable seizures (fits) and kidney failure.

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

At the time of designation, tuberous sclerosis affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 people<sup>\*</sup>, 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, the medicine Votubia (everolimus) was authorised in the EU for the treatment of certain tumours caused by tuberous sclerosis. Other treatments such as vigabatrin and corticotropin were used for treating seizures in patients with tuberous sclerosis.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with tuberous sclerosis because early studies have found that it reduced seizures in patients whose condition could not be controlled by anticonvulsant medicines. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

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<sup>\*</sup>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 515,700,000 (Eurostat 2017).



## How is this medicine expected to work?

Cannabidiol is a substance derived from the *Cannabis sativa* plant. It is expected to work by reducing the activity of mTOR, a protein that is overactive in patients with tuberous sclerosis and is believed to be the cause of many, if not all, features of the disease.

Cannabidiol may also work through other targets to reduce blood supply to the tumours and tumour growth as well as targets on nerve cells to reduce or prevent seizures.

## 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 cannabidiol in patients with tuberous sclerosis were ongoing.

At the time of submission, cannabidiol was not authorised anywhere in the EU for tuberous sclerosis. Orphan designation of the medicine had been granted in the US for tuberous sclerosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 December 2017 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cannabidiol	Treatment of tuberous sclerosis
Bulgarian	Канабидиол	Лечение на туберозна склероза
Croatian	Kanabidiol	Liječenje tuberozne skleroze
Czech	Kanabidiol	Léčba tuberózní sklerózy
Danish	Cannabidiol	Behandling af tuberøs sklerose
Dutch	Cannabidiol	Behandeling van tubereuze sclerose
Estonian	Kannabidiool	Tuberoosse skleroosi ravi
Finnish	Kannabidioli	Tuberoosiskleroosin hoito
French	Cannabidiol	Traitement de la sclérose tubéreuse
German	Cannabidiol	Behandlung der tuberösen Sklerose
Greek	Κανναβιδιόλη	Θεραπεία της οζώδους σκλήρυνσης
Hungarian	Kannabidiol	Sclerosis tuberosa kezelése
Italian	Cannabidiolo	Trattamento della sclerosi tuberosa
Latvian	Kanabidiols	Tuberozās sklerozes ārstēšana
Lithuanian	Kanabidiolis	Tuberozinės sklerozės gydymas
Maltese	Cannabidiol	Kura tal-isklerosi tuberuża
Polish	Kannabidiol	Leczenie stwardnienia guzowatego
Portuguese	Canabidiol	Tratamento da esclerose tuberosa
Romanian	Canabidiol	Tratamentul sclerozei tuberoase
Slovak	Kanabidiol	Liečba tuberóznejsklerózy
Slovenian	Kanabidiol	zdravljenje tuberozne skleroze
Spanish	Cannabidiol	Tratamiento de la esclerosis tuberosa
Swedish	Cannabidiol	Behandling av tuberös skleros
Norwegian	Kannabidiol	Behandling av tuberøs sklerose
Icelandic	Kannabídíól	Meðferð við hnjóskahersli (tuberous sclerosis)

<sup>1</sup> At the time of designation