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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cannabidiol for the treatment of perinatal asphyxia

On 28 July 2015, orphan designation (EU/3/15/1520) was granted by the European Commission to GW Pharma Ltd, United Kingdom, for cannabidiol for the treatment of perinatal asphyxia.

What is perinatal asphyxia?

Perinatal asphyxia happens when babies are born without enough oxygen in their blood. This is generally due to interruptions of the oxygen supplied by the mother through the placenta or the umbilical cord. Perinatal asphyxia can cause damage to the brain and other organs.

Perinatal asphyxia is a long-term debilitating disease because it can lead to the child being mentally and physically disabled. It is also life threatening, with up to 1 baby in 5 with the condition dying within the first days after birth.

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

At the time of designation, perinatal asphyxia 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 orphan designation, there was no treatment for perinatal asphyxia authorised in the EU. Babies with perinatal asphyxia received supportive treatment, and they were sometimes cooled down to a body temperature lower than normal (hypothermia) for 12 to 72 hours after birth to reduce the extent of the damage caused by the asphyxia.

*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 512,900,000 (Eurostat 2015).

How is this medicine expected to work?

Cannabidiol is a substance that is extracted from the cannabis plant, *Cannabis sativa* but which is considered not to have effects on mood, mental activity or behaviour. Although the way this medicine works in perinatal asphyxia is not clearly understood, cannabidiol is thought to have a protective effect on nerve cells due to its expected anti-inflammatory properties and ability to counteract the effect of toxic products containing oxygen. This is expected to improve the symptoms of the condition.

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, no clinical trials with cannabidiol in patients with perinatal asphyxia had been started.

At the time of submission, cannabidiol, in combination with delta-9-tetrahydrocannabinol, was authorised in some EU countries for the treatment of spasticity in multiple sclerosis.

At the time of submission, cannabidiol was not authorised anywhere in the EU for perinatal asphyxia. Orphan designation of cannabidiol had been granted in the United States for the treatment of neonatal hypoxic ischemic encephalopathy (NHIE).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 June 2015 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:

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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 perinatal asphyxia
Bulgarian	Канабидиол	Лечение на перинатална асфиксия
Croatian	Kanabidiol	Liječenje perinatalne asfiksije
Czech	Kanabidiol	Léčba perinatální asfyxie
Danish	Cannabidiol	Behandling af perinatal asfyksi
Dutch	Cannabidiol	Behandeling van perinatale asfyxie
Estonian	Kannabidiool	Perinataalne asfüksia ravi
Finnish	Kannabidioli	Sikiön hapenpuutteen hoito
French	Cannabidiol	Traitement de l'asphyxie périnatale
German	Cannabidiol	Behandlung der perinatalen Asphyxie
Greek	κανναβιδιόλη	Θεραπεία της περιγεννητικής ασφυξίας
Hungarian	Kannabidiol	Perinatális asphyxia kezelése
Italian	Cannabidiolo	Trattamento dell'asfissia perinatale
Latvian	Kanabidiols	Perinatālās asfiksijas ārstēšana
Lithuanian	Kanabidiolis	Perinatalinės asfiksijos gydymas
Maltese	Cannabidiol	Kura tal-asfissija perinatale
Polish	Kannabidiol	Leczenie zamartwicy okołoporodowej
Portuguese	Canabidiol	Tratamento da asfixia perinatal
Romanian	Canabidiol	Tratamentul asfixiei perinatale
Slovak	Kanabidiol	Liečba perinatálnej asfyxie
Slovenian	Kanabidiol	Zdravljenje perinatalne asfiksije
Spanish	Cannabidiol	Tratamiento de la asfixia perinatal
Swedish	Cannabidiol	Behandling av spädbarns asfyxi
Norwegian	Kannabidiol	Behandling av perinatal asfyksi
Icelandic	Kannabídíól	Meðferð burðarmálsköfnunar

¹ At the time of designation