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Public summary of opinion on orphan designation

Ciclosporin for the treatment of bronchiolitis obliterans syndrome

On 24 May 2017, orphan designation (EU/3/04/210) was granted by the European Commission to PARI Pharma GmbH, Germany, for ciclosporin for the treatment of bronchiolitis obliterans syndrome.

The sponsorship was transferred to Breath Therapeutics GmbH, Germany, in August 2017.

What is bronchiolitis obliterans syndrome?

Bronchiolitis obliterans syndrome is the most common complication affecting patients who have had a lung transplant. Rarely, it may also occur in patients who receive a bone marrow transplant. Bronchiolitis obliterans syndrome occurs when cells of the immune system (the body's natural defences) recognise the patient's lungs as 'foreign' and attack them. This leads to extensive scarring (fibrosis) that obstructs and damages the lungs, leading eventually to death.

Bronchiolitis obliterans syndrome is a debilitating and life-threatening disease due to the progressive damage to the lungs.

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

At the time of designation, bronchiolitis obliterans syndrome affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 52,000 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, no satisfactory methods were authorised in the European Union (EU) specifically for the treatment of bronchiolitis obliterans syndrome. Patients received immunosuppressant medicines that reduce the activity of immune cells, with the aim of reducing the immune system's ability to attack the patient's lungs.

*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).

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How is this medicine expected to work?

The medicine contains ciclosporin, a medicine that has been used in the EU since the 1980s to suppress the immune system after a transplant. It is to be inhaled into the lungs as a mist. The ciclosporin is contained in tiny fat particles called liposomes that help it to be absorbed directly into the affected tissues, where it can reduce the activity of the immune cells attacking the lungs without much effect on the rest of the body. This is expected to reduce the development of scarring and obstruction in the airways, and so improve survival.

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 bronchiolitis obliterans syndrome were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for treatment of bronchiolitis obliterans syndrome.

The medicine had been designated orphan on 29 July 2004 for the treatment of graft rejection after lung transplantation. At the request of the sponsor and having assessed the additional data submitted, the COMP adopted an opinion on 11 May 2017 recommending the change of the orphan condition from graft rejection after lung transplantation to bronchiolitis obliterans syndrome.

Orphan designation of the medicine has been granted in the United States for the treatment of bronchiolitis obliterans.

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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	Ciclosporin (inhalation use)	Treatment of bronchiolitis obliterans syndrome
Bulgarian	Циклоспорин (за инхалаторно приложение)	Лечение на синдром на облитериращ бронхиолит
Croatian	Ciklosporin (za inhalaciju)	Liječenje sindroma obliterirajućeg bronhiolitisa
Czech	Ciclosporin (k inhalaci)	Léčba bronchiolitis obliterans syndromu
Danish	Ciclosporin (til inhalation)	Behandling af bronchiolitis obliterans syndrom
Dutch	Ciclosporine (inhalatie)	Behandeling van bronchiolitis obliterans syndroom
Estonian	Tsüklosporiin (inhalatsiooniks)	Oblitereeriva bronhioliidi sündroomi ravi
Finnish	Siklosporiini (inhalaatioon)	Obliteroiva bronkioliitti-oireyhtymän hoito
French	Ciclosporine (voie inhalée)	Traitement de la bronchiolite oblitérante
German	Ciclosporin (zur Inhalation)	Behandlung von Bronchiolitis obliterans Syndrom
Greek	Κυκλοσπορίνη (Χρήση δια εισπνοής)	Θεραπεία του συνδρόμου αποφρακτικής βρογχιολίτιδας
Hungarian	Cyclosporin (inhalációs alkalmazásra)	Bronchiolitis obliterans szindróma kezelése
Italian	Ciclosporina (uso inalatorio)	Trattamento della bronchiolite obliterante
Latvian	Ciklosporīns (inhalācijām)	Obliterējoša bronhiolīta sindroma ārstēšana
Lithuanian	Ciklosporinas (inhaliacijoms)	Obliteruojančio bronchiolito sindromo gydymas
Maltese	Ciclosporin (għal biex jinġibed man-nifs)	Kura tas-sindromu ta' bronkite obliterans
Polish	Cyklosporyna (inhalacja)	Leczenie zespołu zarostowego zapalenia oskrzelików
Portuguese	Ciclosporina (via inalatoria)	Tratamento da síndrome de bronquiolite obliterante
Romanian	Ciclosporină (administrare inhalatorie)	Tratamentul bronşiolitei obliterante
Slovak	Cyklosporín (inhalácia)	Liečba syndrómu bronchiolotis obliterans
Slovenian	Ciklosporin (za inhalacijo)	Zdravljenje obliteracijskega bronhiolitisa
Spanish	Ciclosporina (vía inhalatoria)	Tratamiento de la Bronquiolitis Obliterante
Swedish	Ciclosporin (användning för inhalation)	Behandling av bronkiolitis obliterans-syndrom
Norwegian	Ciklosporin (til inhalasjon)	Behandling av bronkiolitis obliterans syndrom
Icelandic	Ciklósporín (til innöndunar)	Til meðferðar við heilkenni stíflumyndandi berkjubólgu

[1] At the time of designation