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EMA/COMP/89532/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Caffeine citrate for the prevention of bronchopulmonary dysplasia

On 11 April 2014, orphan designation (EU/3/14/1261) was granted by the European Commission to Viridian Pharma Ltd, United Kingdom, for caffeine citrate for the prevention of bronchopulmonary dysplasia.

What is bronchopulmonary dysplasia?

Bronchopulmonary dysplasia is a lung disease affecting premature babies who have been on prolonged mechanical ventilation (using a machine that supplies oxygen to help with breathing). In premature babies the lungs are immature and do not have enough surfactant, which is a substance lining the lungs and airways that allows them to expand properly. Bronchopulmonary dysplasia is caused by the constant high pressure of the oxygen supplied through mechanical ventilation, which causes inflammation and injury to the lungs and blocks the normal development of the alveoli (air sacs in the lungs). This leads to difficulty breathing and weight loss.

Bronchopulmonary dysplasia is a long-term and life-threatening condition because the underdeveloped lungs may not work properly.

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 bronchopulmonary dysplasia was estimated to be between 1 and 3 people in 10,000 in the European Union (EU). This was equivalent to between 51,000 and 153,000 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 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 511,100,000 (Eurostat 2014).

What methods of prevention are available?

At the time of designation, no satisfactory methods of prevention were authorised in the EU for bronchopulmonary dysplasia.

How is this medicine expected to work?

Caffeine citrate is a stimulant of the nervous system and is currently authorised in the EU for the treatment of premature newborns with a breathing disorder called primary apnoea. In the prevention of bronchopulmonary dysplasia, caffeine is expected to act mainly by stimulating the part of the brain that controls breathing, which is thought to help babies breathe on their own and reduce the time spent on mechanical ventilation. In addition, caffeine citrate may also have other effects in the lungs, such as reducing inflammation. These actions are expected to prevent the damage to the lungs in bronchopulmonary dysplasia.

What is the stage of development of this medicine?

The effects of caffeine citrate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with caffeine citrate in patients with bronchopulmonary dysplasia were ongoing.

At the time of submission, caffeine citrate was not authorised anywhere in the EU for bronchopulmonary dysplasia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2014 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	Caffeine citrate	Prevention of bronchopulmonary dysplasia
Bulgarian	Кофеин цитрат	Предотвратяване на бронхопулмонална дисплазия
Croatian	Kofein citrat	Prevenција bronhopulmonalne displazije
Czech	Citrát kofeinu	Prevence bronchopulmonální dysplasie
Danish	Koffeincitrat	Forebyggelse af bronkopulmonal dysplasi
Dutch	Cafeïnecitraat	Preventie van bronchopulmonale dysplasie
Estonian	Kofeiintsitraat	Bronhopulmonaarse düsplaasia ennetamine
Finnish	Kofeiinisitraatti	Bronkopulmonaarisien dysplasian ehkäisy
French	Citrate de caféine	Prévention de la dysplasie broncho-pulmonaire
German	Koffeincitrat	Prävention von bronchopulmonaler Dysplasie
Greek	Κιτρική καφεΐνη	Πρόληψη της βρογχοπνευμονικής δυσπλασίας
Hungarian	Koffein citrát	Bronchopulmonalis dysplasia megelőzése
Italian	Citrato di caffeina	Prevenzione della displasia broncopulmonare
Latvian	Kofeīna citrāts	Bronhopulmonālās displāzijas novēršana
Lithuanian	Kofeino citratas	Bronchopulmoninės displazijos prevencija
Maltese	Caffeine citrate	Prevenzjoni ta' displasja bronkopulmonari
Polish	Kofeiny cytrynian	Zapobieganie dysplazji oskrzelowo-płucnej
Portuguese	Citrato de cafeína	Prevenção de displasia broncopulmonar
Romanian	Citrat de cafeină	Prevenirea displaziei bronhopulmonare
Slovak	Citrát kofeínu	Prevenčia bronchopulmonálnej dysplázie
Slovenian	Kofein citrat	Preprečevanje bronhopulmonalne displazije
Spanish	Citrato de cafeína	Prevención de la displasia broncopulmonar
Swedish	Koffeincitrat	Prevention av bronkopulmonell dysplasi
Norwegian	Koffeinsitrat	Forebygging av bronkopulmonal dysplasi
Icelandic	Koffín sítrat	Til að fyrirbyggja berkjulungna misproska

¹ At the time of designation