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EMA/COMP/443084/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Retinol for the prevention of bronchopulmonary dysplasia

On 22 August 2014, orphan designation (EU/3/14/1307) was granted by the European Commission to Dr Philipp Heinrich Novak, Austria, for retinol 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 damaged and 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 a total of 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).

What methods of prevention are available?

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

*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).

How is this medicine expected to work?

Retinol is the main form of vitamin A, which is known to play an essential role in the growth and development of the lungs, including the formation of the alveoli, and which is found in lower than normal levels in premature infants. Restoring normal levels of retinol in premature babies is expected to improve lung development and help them to absorb oxygen better, thus reducing the need for mechanical ventilation and helping to prevent bronchopulmonary dysplasia. The medicine contains retinol in a form that can be given by injection, as very premature babies cannot absorb vitamin A properly if it is given by mouth.

Medicines containing vitamin A have been available in the EU for many years to treat deficiency of the vitamin.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients at risk of bronchopulmonary dysplasia had been started.

At the time of submission, retinol 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 10 July 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

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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	Retinol	Prevention of bronchopulmonary dysplasia
Bulgarian	Ретинол	Предотвратяване на бронхопулмонална дисплазия
Croatian	Retinol	Prevenција bronhopulmonalne displazije
Czech	Retinol	Prevence bronchopulmonální dysplasie
Danish	Retinol	Forebyggelse af bronkopulmonal dysplasi
Dutch	Retinol	Preventie van bronchopulmonale dysplasie
Estonian	Retinool	Bronhopulmonaarse düsplaasia ennetamine
Finnish	Retinoli	Bronkopulmonaarisien dysplasian ehkäisy
French	Rétinol	Prévention de la dysplasie broncho-pulmonaire
German	Retinol	Prävention von bronchopulmonaler Dysplasie
Greek	Ρετινόλη	Πρόληψη της βρογχοπνευμονικής δυσπλασίας
Hungarian	Retinol	Bronchopulmonalis dysplasia megelőzése
Italian	Retinolo	Prevenzione della displasia broncopulmonare
Latvian	Retinols	Bronhopulmonālās displāzijas novēršana
Lithuanian	Retinolis	Bronchopulmoninės displazijos prevencija
Maltese	Retinol	Prevenzjoni ta' displasja bronkopulmonari
Polish	Retinol	Zapobieganie dysplazji oskrzelowo-płucnej
Portuguese	Retinol	Prevenção de displasia broncopulmonar
Romanian	Retinol	Prevenirea displaziei bronhopulmonare
Slovak	Retinol	Prevenčia bronchopulmonálnej dysplázie
Slovenian	Retinol	Preprečevanje bronhopulmonalne displazije
Spanish	Retinol	Prevención de la displasia broncopulmonar
Swedish	Retinol	Prevention av bronkopulmonell dysplasi
Norwegian	Retinol	Forebygging av bronkopulmonal dysplasi
Icelandic	Retínól	Til að fyrirbyggja berkjulungna misþroska

¹ At the time of designation