



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 November 2020
EMADOC-628903358-2622

Public summary of opinion on orphan designation

Retinol palmitate for the prevention of bronchopulmonary dysplasia

On 27 July 2020, orphan designation EU/3/20/2306 was granted by the European Commission to Provepharm S.A.S, France, for retinol palmitate 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 (to push oxygen-rich air into the lungs). The constant high pressure of the oxygen from mechanical ventilation can cause inflammation and damage to the cells in the lungs and hinder normal development of the lungs. This can lead to long-term breathing problems and weight loss.

Bronchopulmonary dysplasia is a long-term and life-threatening condition because of the damage to the lungs, leading to lack of oxygen in the blood.

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 less than 2 people in 10,000 in the European Union (EU). This was equivalent to a total of fewer than 104,000 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 were authorised in the EU for the prevention of bronchopulmonary dysplasia.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).



How is this medicine expected to work?

Retinol, a form of vitamin A, plays an essential role in the growth and development of the lungs. Premature babies have low levels of retinol. Restoring normal levels of retinol is expected to improve lung development and help the lungs 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?

The effects of retinol palmitate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with retinol palmitate in patients with bronchopulmonary dysplasia had finished.

At the time of submission, medicines containing retinol palmitate were authorised in the EU for the treatment of vitamin A deficiency. However, retinol palmitate was not authorised anywhere in the EU for the prevention of bronchopulmonary dysplasia or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 June 2020, 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

Contact details of the current sponsor for this orphan designation can be found on [EMA website](#).

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 palmitate	Prevention of bronchopulmonary dysplasia
Bulgarian	Ретинол палмитат	Предотвратяване на бронхопулмонална дисплазия
Croatian	Retinol palmitat	Prevenција bronhopulmonalne displazije
Czech	Retinol palmitát	Prevence bronchopulmonální dysplasie
Danish	Retinolpalmitat	Forebyggelse af bronkopulmonal dysplasi
Dutch	Retinolpalmitaat	Preventie van bronchopulmonale dysplasie
Estonian	Retinoolpalmitaat	Bronhopulmonaarse düsplaasia ennetamine
Finnish	Retinolipalmitaatti	Bronkopulmonaarisien dysplasian ehkäisy
French	Palmitate de rétinol	Prévention de la dysplasie broncho-pulmonaire
German	Retinolpalmitat	Prävention von bronchopulmonaler Dysplasie
Greek	Παλμιτική ρετινόλη	Πρόληψη της βρογχοπνευμονικής δυσπλασίας
Hungarian	Retinol-palmitát	Bronchopulmonalis dysplasia megelőzése
Italian	Palmitato di retinolo	Prevenzione della displasia broncopulmonare
Latvian	Retinola palmitāts	Bronhopulmonālās displāzijas novēršana
Lithuanian	Retinolio palmitatas	Bronchopulmoninės displazijos prevencija
Maltese	Palmitat tar-retinol	Prevenzjoni ta' displasja bronkopulmonari
Polish	Palmitynian retinolu	Zapobieganie dysplazji oskrzelowo-płucnej
Portuguese	Palmitato de retinol	Prevenção de displasia broncopulmonar
Romanian	Palmitat de retinol	Prevenirea displaziei bronhopulmonare
Slovak	Retinol palmitát	Prevenia bronchopulmonálnej dysplázie
Slovenian	Retinol palmitat	Preprečevanje bronhopulmonalne displazije
Spanish	Palmitato de retinol	Prevención de la displasia broncopulmonar
Swedish	Retinolpalmitat	Prevention av bronkopulmonell dysplasi
Norwegian	Retinolpalmitat	Forebygging av bronkopulmonal dysplasi
Icelandic	Retínólpalmitat	Til að fyrirbyggja berkjulungna misþroska

¹ At the time of designation