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Public summary of opinion on orphan designation

Recombinant fragment of human surfactant protein-D for the prevention of bronchopulmonary dysplasia

On 23 August 2017, orphan designation (EU/3/17/1907) was granted by the European Commission to Trimunocor Ltd, United Kingdom, for recombinant fragment of human surfactant protein-D 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 artificially 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 the 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 103,000 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.

*Disclaimer: For the purpose of the designation, the number of patients at risk of developing 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?

This medicine consists of a fragment of surfactant protein-D, a protein found in the lungs that helps reduce inflammation. By giving the medicine in the first few days of life, it is expected to overcome the deficiency of surfactant protein-D in premature babies and so prevent the inflammatory process that can cause bronchopulmonary dysplasia.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

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

At the time of submission, the medicine 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 13 July 2017 recommending the granting of this designation.

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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	Recombinant fragment of human surfactant protein-D	Prevention of bronchopulmonary dysplasia
Bulgarian	Рекомбинантен фрагмент от протеин D на човешкия сърфактант	Предотвратяване на бронхопулмонална дисплазия
Croatian	Rekombinantni fragment ljudskog surfaktantog proteina D	Prevencija bronhopulmonalne displazije
Czech	Rekombinantní fragment proteinu D lidského surfaktantu	Prevence bronchopulmonální dysplasie
Danish	Rekombinant fragment humant surfactant-protein D	Forebyggelse af bronkopulmonal dysplasi
Dutch	Recombinant humaan fragment surfactant proteïne D	Preventie van bronchopulmonale dysplasie
Estonian	Inimese surfaktantvalgu D rekombinantne fragment	Bronhopulmonaarse düsplaasia ennetamine
Finnish	Ihmisen surfaktanttiproteiini D:n rekombinantti fragmentti	Bronkopulmonaarisen dysplasian ehkäisy
French	Protéine surfactante humaine de fragment recombinant D	Prévention de la dysplasie broncho-pulmonaire
German	Rekombinantes Fragment des menschlichen Tensidprotein D	Prävention von bronchopulmonaler Dysplasie
Greek	Ανασυνδυασμένο τμήμα της επιφανειοδραστικής πρωτεΐνης D	Πρόληψη της βρογχοπνευμονικής δυσπλασίας
Hungarian	Rekombináns humán surfactant protein-D fragmentum	Bronchopulmonalis dysplasia megelőzése
Italian	Frammento ricombinante della proteina D del surfattante umano	Prevenzione della displasia broncopolmonare
Latvian	Rekombinants cilvēka surfaktanta proteīna D fragments	Bronhopulmonālās displāzijas novēršana
Lithuanian	Žmogaus surfaktanto baltymo-D rekombinantinis fragmentas	Bronchopulmoninės displazijos prevencija
Maltese	Framment rikombinanti tal-proteina umana D tas-surfaktant	Prevenzjoni ta' displasja bronkopulmonari
Polish	Rekombinowany fragment ludzkiego białka surfaktantu D	Zapobieganie dysplazji oskrzelowo-płucnej
Portuguese	Fragmento recombinante da proteína-D surfactante humana	Prevenção da displasia broncopulmonar
Romanian	Fragment recombinant al proteinei D a surfactantului uman	Prevenirea displaziei bronhopulmonare
Slovak	Rekombinantný ľudský fragment surfaktačného proteínu D	Prevencia bronchopulmonálnej dysplázie

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Rekombinantni fragment humanega surfaktanta protein D	Preprečevanje bronhopulmonalne displazije
Spanish	Proteína surfactante humana de fragmento recombinante D	Prevención de la displasia broncopulmonar
Swedish	Rekombinant fragment humant surfaktantprotein D	Prevention av bronkopulmonell dysplasi
Norwegian	Rekombinant fragment humant surfaktant protein D	Forebygging av bronkopulmonal dysplasi
Icelandic	Raðbrigða brot af manna yfirborðs próteini D	Til að fyrirbyggja berkjulungna misþroska