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EMA/COMP/92638/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human surfactant protein D for the prevention of bronchopulmonary dysplasia

On 11 April 2014, orphan designation (EU/3/14/1262) was granted by the European Commission to Dr Ulrich Granzer, Germany, for recombinant 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 (using a machine that supplies oxygen to help with breathing). In premature babies the lungs are immature and do not have enough surfactant, 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

*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?

Recombinant human surfactant protein D is one of the components of the natural surfactant present in the lungs. The medicine is expected to work by replacing the missing surfactant protein D in the lungs of premature infants who are at risk of developing bronchopulmonary dysplasia. This should improve the child's breathing function by reducing inflammation to the lungs and allowing sufficient oxygen to be taken up.

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 with 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 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	Recombinant human surfactant protein D	Prevention of bronchopulmonary dysplasia
Bulgarian	Рекомбинантен човешки сурфактантен протеин D	Предотвратяване на бронхопулмонална дисплазия
Croatian	Rekombinantni ljudski surfaktant protein D	Prevenција bronhopulmonalne displazije
Czech	Rekombinantní lidský surfaktantový protein D	Prevence bronchopulmonální dysplasie
Danish	Rekombinant humant surfactant-protein D	Forebyggelse af bronkopulmonal dysplasi
Dutch	Recombinant humaan surfactant proteïne D	Preventie van bronchopulmonale dysplasie
Estonian	Rekombinantne inimese surfaktantvalk D	Bronhopulmonaarse düsplaasia ennetamine
Finnish	Yhdistelmä-DNA-tekniikalla valmistettu ihmisen surfaktanttiproteiini D	Bronkopulmonaarisien dysplasian ehkäisy
French	Protéine D recombinante humaine du surfactant	Prévention de la dysplasie broncho-pulmonaire
German	Rekombinantes humanes Surfactant-Protein D	Prävention von bronchopulmonaler Dysplasie
Greek	Ανασυνδυασμένη ανθρώπινη πρωτεΐνη D του επιφανειοδραστικού παράγοντα	Πρόληψη της βρογχοπνευμονικής δυσπλασίας
Hungarian	Humán rekombináns surfaktant protein-D	Bronchopulmonalis dysplasia megelőzése
Italian	Proteina D ricombinante del surfattante umano	Prevenzione della displasia broncopulmonare
Latvian	Rekombinants cilvēka surfaktanta proteīns D	Bronhopulmonālās displāzijas novēršana
Lithuanian	Rekombinantinis žmogaus surfaktanto baltymas D	Bronchopulmoninės displazijos prevencija
Maltese	Proteina D tas- <i>surfactant</i> rikombinanti umana	Prevenzjoni ta' displasja bronkopulmonari
Polish	Rekombinowane białko D ludzkiego surfaktantu	Zapobieganie dysplazji oskrzelowo-płucnej
Portuguese	Proteína D recombinante humana do surfactante	Prevenção de displasia broncopulmonar
Romanian	Proteina D umană recombinantă a surfactantului	Prevenirea displaziei bronhopulmonare
Slovak	Rekombinantný proteín D ľudského surfaktantu	Prevenčia bronchopulmonálnej dysplázie

¹ At the time of designation

Language	Active ingredient	Indication
Slovenian	Rekombinantni protein D humanega surfaktanta	Preprečevanje bronhopulmonalne displazije
Spanish	Proteína D tensioactiva humana recombinante	Prevención de la displasia broncopulmonar
Swedish	Rekombinant humant surfaktantprotein D	Prevention av bronkopulmonell dysplasi
Norwegian	Rekombinant humant overflateaktivt protein D	Forebygging av bronkopulmonal dysplasi
Icelandic	Raðbrigða manna yfirborðsvirkt prótein D	Til að fyrirbyggja berkjulungna misþroska