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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic ex-vivo-expanded human umbilical cord blood-derived mesenchymal stem cells for the prevention of bronchopulmonary dysplasia

On 19 June 2015, orphan designation (EU/3/15/1503) was granted by the European Commission to PSR Group B.V., the Netherlands, for allogeneic ex-vivo-expanded human umbilical cord blood-derived mesenchymal stem cells 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 drive 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, as well as 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 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 154,000 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 512,900,000 (Eurostat 2015).

How is this medicine expected to work?

This medicine is made of stem cells extracted from umbilical cord blood donated after birth and then grown in a laboratory, in order to increase their numbers. Although it is not fully understood how they work, when these cells are given to premature babies at risk of bronchopulmonary dysplasia, they could potentially mature and replace the damaged cells in the tissue of the lungs. The medicine is expected to reduce the activity of the immune cells that cause inflammation and damage to the lungs.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients at high risk of bronchopulmonary dysplasia were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for bronchopulmonary dysplasia. Orphan designation of the medicine had been granted in the United States for the prevention of this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 May 2015 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	Allogeneic ex-vivo-expanded human umbilical cord blood-derived mesenchymal stem cells	Prevention of bronchopulmonary dysplasia
Bulgarian	Алогенни ex vivo култивирани човешки мезенхимни стволови клетки от умбиликална кръв	Предотвратяване на бронхопулмонална дисплазия
Croatian	Alogene ex-vivo umnožene ljudske mezenhimalne matične stanice izvedene iz krvi pupčane vrpce	Prevenција bronhopulmonalne displazije
Czech	Alogenní ex vivo kultivované mezenchymální kmenové buňky získané z lidské pupečnickové krve	Prevence bronchopulmonální dysplasie
Danish	Allogene ex vivo ekspanderede mesenchymale stamceller afledt af human navlestrengsblod	Forebyggelse af bronkopulmonal dysplasi
Dutch	Allogene ex vivo geëxpandeerde humane navelstrengbloed-afgeleide mesenchymale stamcellen	Preventie van bronchopulmonale dysplasie
Estonian	Allogeensed ex vivo kasvatatud inimese nabaväädiverest lähtunud mesenhümaalsed tüvirakud	Bronhopulmonaarse düsplaasia ennetamine
Finnish	Allogeeniset ex vivo kasvatetut, ihmisen napaverestä peräisin olevat mesenkymaaliset kantasolut	Bronkopulmonaarisien dysplasian ehkäisy
French	Cellules souches mésenchymateuses allogéniques dérivées du sang de cordon ombilical humain amplifiées ex-vivo	Prévention de la dysplasie broncho-pulmonaire
German	Allogene, ex vivo expandierte, aus humanem Nabelschnurblut gewonnene mesenchymale Stammzellen	Prävention von bronchopulmonaler Dysplasie
Greek	Αλλογενή ex-vivo καλλιεργημένα μεσεγχυματικά βλαστικά κύτταρα που προέρχονται από αίμα ανθρώπινου ομφάλιου λώρου	Πρόληψη της βρογχοπνευμονικής δυσπλασίας
Hungarian	Allogén, ex-vivo expandiált, humán köldökszínórvérből származó mesenchimális őssejtek	Bronchopulmonalis dysplasia megelőzése
Italian	Cellule staminali allogeniche mesenchimali derivate da sangue cordonale umano, espanse ex-vivo	Prevenzione della displasia broncopulmonare
Latvian	Allogēnas ex vivo pavairotais no cilvēka nabas saitēs asinīm iegūtas mezenhimālās cilmes šūnas	Bronhopulmonālās displāzijas novēršana
Lithuanian	Aloeninės, ex vivo pagausintos, iš žmogaus virkštelės kraujo išskirtos kamieninės mezenchimos ląstelės	Bronchopulmoninės displazijos prevencija
Maltese	Ċelluli staminali mesenkimali alloġeniċi mnissla mid-dem ta-kurdun uman, mwassa' ex vivo	Prevenzjoni ta' displasja bronkopulmonari
Polish	Allogeniczne mezenchymalne komórki macierzyste uzyskane z ludzkiej krwi pępowinowej namnożone ex vivo	Zapobieganie dysplazji oskrzelowo-płucnej

¹ At the time of designation

Language	Active ingredient	Indication
Portuguese	Células alogénicas estaminais mesenquimais derivadas de sangue do cordão umbilical humano expandidas ex vivo	Prevenção da displasia broncopulmonar
Romanian	Celule stem alogenice mezenchimale derivate din sângele din cordonul ombilical uman amplificate ex vivo	Prevenirea displaziei bronhopulmonare
Slovak	Alogénne ex vivo rozšírené mezenchymálne kmeňové bunky z ľudskej pupočníkovej krvi	Prevenia bronchopulmonálnej dysplázie
Slovenian	Alogenske, ex vivo ekspandirane, mezenhimske matične celice, pridobljene iz človeške umbilikalne krvi	Preprečevanje bronhopulmonalne displazije
Spanish	Células madre mesenquimales alogénicas provenientes de sangre de cordón umbilical humano expandidas ex vivo	Prevención de la displasia broncopulmonar
Swedish	Allogeniska ex vivo expanderade humana mesenkymala stamceller från navelsträngsblod	Prevention av bronkopulmonell dysplasi
Norwegian	Allogene ex vivo ekspanderte humane mesenchymal-stamceller fra navlestrengsblod	Forebygging av bronkopulmonal dysplasi
Icelandic	Ósamgena ex vivo útvíkkaðar bandvefskímstofnfrumur fengnar úr naflastrengsblóði manna	Til að fyrirbyggja berkjulungna misþroska