



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Allogeneic umbilical cord tissue-derived mesenchymal stromal cells ex vivo expanded for the prevention of bronchopulmonary dysplasia

On 21 August 2020, orphan designation EU/3/20/2308 was granted by the European Commission to MDTB Cells GmbH, Germany, for allogeneic umbilical cord tissue-derived mesenchymal stromal cells ex vivo expanded 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 approximately 2.6 people in 10,000 in the European Union (EU). This was equivalent to a total of around 135,000 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?

The medicine contains cells called mesenchymal stromal cells (MSCs) obtained from donor umbilical cord and grown in a laboratory to increase their numbers. MSCs are thought to increase production of substances that prevent inflammation and oxygen-induced damage, protect lung cells and encourage the repair and development of lung tissue. This is expected to prevent the symptoms of bronchopulmonary dysplasia.

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, no clinical trials with the medicine in patients with bronchopulmonary dysplasia had been started.

At the time of submission, allogeneic umbilical cord tissue-derived mesenchymal stromal cells ex vivo expanded were 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 16 July 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	Allogeneic umbilical cord tissue-derived mesenchymal stromal cells ex vivo expanded	Prevention of bronchopulmonary dysplasia
Bulgarian	Алогенни мезенхимни стромални клетки култивирани ex vivo от пъпната връв	Предотвратяване на бронхопулмонална дисплазия
Croatian	Alogene ex vivo ekspandirane ex-vivo umnožene mezenhimalne stromalne stanice izvedene iz tkivo tkiva pupčane vrpce	Prevenција bronhopulmonalne displazije
Czech	Alogenní mezenchymální stromální buňky získané z pupečnickové tkáně, ex vivo expandované	Prevence bronchopulmonální dysplasie
Danish	Allogene ex vivo ekspanderede mesenkymale stromale celler afledt af navlestreng væv	Forebyggelse af bronkopulmonal dysplasi
Dutch	Allogene ex vivo geëxpandeerde navelstrengweefsel-afgeleide mesenchymale stromale cellen	Preventie van bronchopulmonale dysplasie
Estonian	Allogeensed nabaväädikoest pärinevad ja ex vivo paljundatud mesenhümaalsed stroomarakud	Bronhopulmonaarse düsplaasia ennetamine
Finnish	Allogeeniset ex vivo kasvatetut, napanuora kudoksesta peräisin olevat mesenkymaaliset stroomasolut	Bronkopulmonaarisen dysplasian ehkäisy
French	Cellules stromales mésenchymateuses allogéniques dérivées du tissu de cordon ombilical expanses ex-vivo	Prévention de la dysplasie broncho-pulmonaire
German	Allogene, ex vivo expandierte, aus Nabelschnurgewebe gewonnene mesenchymale Stromazellen	Prävention von bronchopulmonaler Dysplasie
Greek	Αλλογενή ex-vivo πολλαπλασιασμένα μεσεγχυματικά στρωματικά κύτταρα που προέρχονται από ιστό ομφάλιου λώρου	Πρόληψη της βρογχοπνευμονικής δυσπλασίας
Hungarian	Allogén, ex-vivo expandált, köldökszínór szövetéből származó mesenchymális stroma sejtek	Bronchopulmonalis dysplasia megelőzése
Italian	Cellule stromali allogeniche mesenchimali derivate da tessuto cordonale, espanse ex-vivo	Prevenzione della displasia broncopulmonare
Latvian	Allogēnas ex vivo pavairotas no nabas saites audiem iegūtas mezenhimālās stromas šūnas	Bronhopulmonālās displāzijas novēršana

¹ At the time of designation

Language	Active ingredient	Indication
Lithuanian	Aloeninės mezenchimos stromos ląstelės, išskirtos iš virkštelės audinio ir pagausintos <i>ex vivo</i> ląstelės	Bronchopulmoninės displazijos prevencija
Maltese	Ċelloli stromali mesenkimali alloġeniċi mniġssla mit-tessut tal-kurdun umbiliku mwessa' <i>ex vivo</i>	Prevenzjoni ta' displasja bronkopulmonari
Polish	Allogeniczne mezenchymalne komórki zrębu uzyskane z tkanki pępowinowej namnożone <i>ex vivo</i>	Zapobieganie dysplazji oskrzelowo-płucnej
Portuguese	Células estromais mesenquimais alogénicas derivadas do tecido do cordão umbilical expandidas <i>ex vivo</i>	Prevenção da displasia broncopulmonar
Romanian	Celule stromale alogenice mezenchimale derivate din tesut din cordonul ombilical expandate <i>ex vivo</i>	Prevenirea displaziei bronhopulmonare
Slovak	Alogénne <i>ex vivo</i> expandované mezenchymálne stromálne bunky z pupočníkového tkaniva	Prevenia bronchopulmonálnej dysplázie
Slovenian	Alogenske, <i>ex vivo</i> ekspandirane mezenhimske stromalne celice, pridobljene iz tkiva popkovnice	Preprečevanje bronhopulmonalne displazije
Spanish	Células estromales mesenquimales alogénicas provenientes de tejido de cordón umbilical expandidas <i>ex vivo</i>	Prevención de la displasia broncopulmonar
Swedish	Allogena <i>ex-vivo</i> expanderade mesenkymala stromala celler från navelsträngsvävnad	Prevention av bronkopulmonell dysplasi
Norwegian	Allogene mesenkymale stromale celler fra navlestrengsvev ekspandert <i>ex vivo</i>	Forebygging av bronkopulmonal dysplasi
Icelandic	Ósamgena bandvefsstofnfrumur úr naflastrengsvef, fjölgað <i>ex vivo</i>	Til að fyrirbyggja berkjulungna misþroska