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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous mononuclear cells derived from human cord blood for the treatment of periventricular leukomalacia

On 14 October 2016, orphan designation (EU/3/16/1744) was granted by the European Commission to BrainRepair UG (haftungsbeschränkt), Germany, for autologous mononuclear cells derived from human cord blood for the treatment of periventricular leukomalacia.

What is periventricular leukomalacia?

Periventricular leukomalacia is a type of brain damage that occurs in premature babies because of a lack of oxygen or blood in parts of the brain ('white matter'). The condition is not usually noticeable at birth, but signs of brain damage may start to show in early childhood.

Periventricular leukomalacia is life-threatening and debilitating in the long term because of the risk of the child developing problems with learning, sight, behaviour and movement.

What is the estimated number of patients affected by the condition?

At the time of designation, periventricular leukomalacia affected approximately 0.3 in 10,000 people per year in the European Union (EU). This was equivalent to a total of around 15,000 people per year*, and is below the ceiling for orphan designation. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of the orphan designation there were no satisfactory treatments authorised for periventricular leukomalacia in the EU.

*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 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

This medicine is a type of advanced therapy medicine called a 'tissue engineered product'. This is a type of medicine containing cells or tissues that have been manipulated so that they can be used to repair, regenerate or replace tissue.

The medicine is made of 'mononuclear cells', which include immune cells and stem cells (cells that can develop into different types of cell), from the blood in the baby's umbilical cord. At birth, the cord blood is collected and the mononuclear cells are harvested and stored, so that they are ready to be injected back into the baby should periventricular leukomalacia occur. When injected, the mononuclear cells are expected to move to the oxygen-poor damaged brain region, where they release cytokines (messenger molecules of the immune system) and other substances, which in turn are expected to improve survival of brain cells. This is expected to improve the symptoms of the condition.

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 periventricular leukomalacia had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for periventricular leukomalacia 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 8 September 2016 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	Autologous mononuclear cells derived from human cord blood	Treatment of periventricular leukomalacia
Bulgarian	Автоложни мононуклеарни клетки получени от човешка пъпна кръв	Лечение на перивентрикуларна левкомалация
Croatian	Autologne mononuklearne stanice dobivene iz krvi pupkovine čovjeka	Liječenje periventrikularne leukomalacije
Czech	Autologní mononukleární buňky pupečníkové krve	Léčba periventrikulární leukomalacie
Danish	Autologe mononukleære celler afledt fra humant navlesnor blod	Behandling af periventrikulær leukomalaci
Dutch	Autologe mononucleaire cellen afgeleid uit humaan navelstrengbloed	Behandeling van periventriculaire leukomalacia
Estonian	Inimese nabaväädiverest lähtuvad autoloogsed mononukleaarsed rakud	Periventrikulaarse leukomalaatsia ravi
Finnish	Autologiset mononukleaariset solut, jotka ovat peräisin ihmisen napaverestä	Periventrikulaarisen leukomalasian hoito
French	Cellules mononucléaires autologues dérivées de sang de cordon humain	Traitement du leucomalacie périventriculaire
German	Autologe mononukleäre Zellen gewonnen aus Nabelschnurblut	Behandlung von periventrikuläre Leukomalazie
Greek	Αυτόλογα μονοπύρηνα κύτταρα απομονωμένα από ομφαλοπλακουντικό αίμα	Θεραπεία της περικοιλιακής λευκομαλακίας
Hungarian	Humán köldökzsinórvérből származó autológ mononukleáris sejtek	Periventricularis leukomalacia kezelésére
Italian	Cellule mononucleari autologhe derivateda sangue di cordone ombelicale umano	Trattamento del leucomalacia periventricolare
Latvian	Autologas mononukleāras šūnas, kas iegūtas no cilvēka nabas saites asinīm	Periventrikulārās leikomalācijas ārstēšana
Lithuanian	Autologinės mononuklearinės ląstelės, išskirtos iš žmogaus virkštelės kraujo	Periventrikulinės leukomaliacijos gydymas
Maltese	Ċelluli mononukleari awtologużi mnissla minn demm tal-kurdun uman	Kura tal-lewkomalaċa periventrikulari
Polish	Autologiczne komórki mononuklearne wywodzące się z ludzkiej krwi pępowinowej	Leczenie leukomalacji okołokomorowej
Portuguese	Células mononucleares autólogas derivadas de sangue do cordão umbilical humano	Tratamento da leucomalácia periventricular

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Celule mononucleare autologe derivate din sângele cordonului ombilical uman	Tratamentul leucomalaciei periventriculare
Slovak	Autológne mononukleárne bunky z krvi ľudskej pupočnej šnúry	Liečba periventrikulárna leukomalácia
Slovenian	Avtologne mononuklearne celice pridobljene iz humane popkovnične krvi	Zdravljenje periventrikularna levkomalacija
Spanish	Celulas mononucleares autologas derivadas de sangre de cordon.	Tratamiento del leucomalacia periventricular
Swedish	Autologa mononukleära celler från humant navelsträngsblod	Behandling av periventrikulär leukomalasi
Norwegian	Autologe mononukleære celler fra humant navlestrengsblod	Behandling av periventrikulær leukomalasi
Icelandic	Samgena einkjarna frumur úr manna naflastrengsblóði	Meðferð periventricular leukómalacíu