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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 neonatal encephalopathy

On 14 October 2016, orphan designation (EU/3/16/1743) was granted by the European Commission to BrainRepair UG (haftungsbeschränkt), Germany, for autologous mononuclear cells derived from human cord blood for the treatment of neonatal encephalopathy.

What is neonatal encephalopathy?

Neonatal encephalopathy refers to brain damage that occurs around the time of birth in babies who are not premature. Symptoms include reduced level of consciousness, seizures (fits), difficulty breathing, low muscle tone and poor reflexes. It is often caused by low levels of oxygen in the blood.

Neonatal encephalopathy is a long-term debilitating disease due to its effects on mental and physical development, and can often be life-threatening in the most severe cases.

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

At the time of designation, neonatal encephalopathy affected approximately 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 15,000 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 treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for treating neonatal encephalopathy. Babies received supportive treatment and were often cooled down to a body temperature lower than normal (hypothermia) to reduce extent of damage.

*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 neonatal encephalopathy 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 in patients with neonatal encephalopathy were planned.

At the time of submission, the medicine was not authorised anywhere in the EU for neonatal encephalopathy 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 neonatal encephalopathy
Bulgarian	Автоложни мононуклеарни клетки получени от човешка пъпна кръв	Лечение на неонатална енцефалопатия
Croatian	Autologne mononuklearne stanice dobivene iz krvi pupkovine čovjeka	Liječenje neonatalne encefalopatije
Czech	Autologní mononukleární buňky pupečníkové krve	Léčba neonatální encefalopatie
Danish	Autologe mononukleære celler afledt fra humant navlesnor blod	Behandling af neonatal encephalopati
Dutch	Autologe mononucleaire cellen afgeleid uit humaan navelstrengbloed	Behandeling van neonatale encephalopathie
Estonian	Inimese nabaväädiverest lähtuvad autoloogsed mononukleaarsed rakud	Neonataalse entsefalopaatia ravi
Finnish	Autologiset mononukleaariset solut, jotka ovat peräisin ihmisen napaverestä	Neonataalisen enkefalopatian hoito
French	Cellules mononucléaires autologues dérivées de sang de cordon humain	Traitement de l'encéphalopathie néonatale
German	Autologe mononukleäre Zellen gewonnen aus Nabelschnurblut	Behandlung der neonatalen Enzephalopathie
Greek	Αυτόλογα μονοπύρηνα κύτταρα απομονωμένα από ομφαλοπλακουντικό αίμα	Θεραπεία της νεογνικής εγκεφαλοπάθειας
Hungarian	Humán köldökzsinórvérből származó autológ mononukleáris sejtek	Újszülöttkori enkefalopátia kezelésére
Italian	Cellule mononucleari autologhe derivateda sangue di cordone ombelicale umano	Trattemento dell'encefalopatia neonatale
Latvian	Autologas mononukleāras šūnas, kas iegūtas no cilvēka nabas saites asinīm	Neonatālas encefalopātijas ārstēšana
Lithuanian	Autologinės mononuklearinės ląstelės, išskirtos iš žmogaus virkštelės kraujo	Naujagimių encefalopatijos gydymas
Maltese	Ċelluli mononukleari awtologużi mnissla minn demm tal-kurdun uman	Kura tal-enċefalopatija fi trabi tat-twelid
Polish	Autologiczne komórki mononuklearne wywodzące się z ludzkiej krwi pępowinowej	Leczenie encefalopatii noworodka
Portuguese	Células mononucleares autólogas derivadas de sangue do cordão umbilical humano	Tratamento da encefalopatia neonatal

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Celule mononucleare autologe derivate din sângele cordonului ombilical uman	Tratamentul encefalopatiei neonatale
Slovak	Autológne mononukleárne bunky z krvi ľudskej pupočnej šnúry	Liečba neonatálnej encefalopatie
Slovenian	Avtologne mononuklearne celice, pridobljene iz humane popkovnične krvi	Zdravljenje neonatalne encefalopatije
Spanish	Celulas mononucleares autologas derivadas de sangre de cordon.	Tratamiento de la encefalopatia neonatal
Swedish	Autologa mononukleära celler från humant navelsträngsblod	Behandling av neonatal encefalit
Norwegian	Autologe mononukleære celler fra humant navlestrengsblod	Behandling av neonatal encefalopati
Icelandic	Samgena einkjarna frumur ú manna naflastrengsblóði	Meðferð nýbura heilakvilla