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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

allogeneic human umbilical cord tissue-derived cells for treatment of retinitis pigmentosa

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in April 2010 on request of the sponsor.

On 1 April 2008, orphan designation (EU/3/08/534) was granted by the European Commission to Centocor, B.V., The Netherlands, for allogeneic human umbilical cord tissue-derived cells for treatment of retinitis pigmentosa.

What is retinitis pigmentosa?

Retinitis pigmentosa is a genetic disorder, characterised by progressive loss of sight. In retinitis pigmentosa, some cells in the retina (the light-sensitive part of the eye), called rods and cones, are progressively damaged and disappear. These cells are fundamental for eyesight. Retinitis pigmentosa is chronically debilitating, due to progressive loss of vision.

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

At the time of designation, retinitis pigmentosa affected approximately 2.9 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 144,000 people, and is below the threshold 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 submission of the application for orphan designation, no satisfactory method had been authorised in the European Union for the treatment of the condition. Treatment of patients with retinitis pigmentosa primarily involved genetic counselling, and general support such as information and regular medical follow up.

[*]Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006).

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How is this medicine expected to work?

Photoreceptors are cells found at the back of the eye in retina (the internal lining of the eye). The function of photoreceptor is fundamental for the perception of light as they are specialised in receiving light and transforming it into nervous signals for the brain. In many patients with retinitis pigmentosa, there is a loss of function of these cells.

The umbilical cord is the tube that connects a foetus to the placenta, and through which the foetus receives oxygenated and nutrient-rich blood during the pregnancy. The general practise is to clamp and cut the umbilical cord some minutes after birth of the child. The medicinal product consists of cells that originate from umbilical cord tissue that is donated (allogenic). The exact mechanism of action of allogeneic human umbilical cord tissue-derived cells is not yet fully characterized but it is believed that these cells are able to maintain the visual function by reducing the rate of the death of photoreceptors.

What is the stage of development of this medicine?

The effects of allogeneic human umbilical cord tissue-derived cells were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with retinitis pigmentosa were ongoing.

Allogeneic human umbilical cord tissue-derived cells was not authorised anywhere worldwide for retinitis pigmentosa, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2008 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 European Union) 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

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Allogeneic human umbilical cord tissue-derived cells	Treatment of retinitis pigmentosa
Bulgarian	Алогенни човешки клетки от пъпна връв	Лечение на пигментен ретинит
Czech	Alogenní buňky získané z tkáně lidského pupečníku	Léčba pigmentosní retitinitidy
Danish	Allogene celler fra humant navlestrengsvæv	Behandling af retinitis pigmentosa
Dutch	Allogene humane cellen bereid uit navelstrengweefsel	Behandeling van retinitis pigmentosa
Estonian	Allogeensed inimese nabaväädii-koest pärinevad rakud	Pigmentoosse võrkkestapõletiku ravi
Finnish	Allogeeniset ihmisen napanuorakudoksesta peräisin olevat solut	Hoito verkkokalvorappeumaan
French	Cellules allogéniques produites à partir de tissu du cordon ombilical humain	Traitement de la rétinite pigmentaire
German	Allogene humane Nabelschnurgewebszellen	Behandlung der Retinopathia Pigmentosa
Greek	Αλλογενή κύτταρα ανθρωπίνου ομφαλίου λώρου	Αγωγή κατά της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Allogén, humán, köldökzsinór-szövet eredetű sejtek	Retinitis pigmentosa kezelése
Italian	Cellule allogeniche derivanti da tessuto del cordone ombelicale umano	Trattamento della retinite pigmentosa
Latvian	Alogēnas šūnas, kas iegūtas no cilvēka nabassaites audiem	Retinitis pigmentosa ārstēšana
Lithuanian	Alogeninės žmogaus ląstelės iš virkštelės audinio	Pigmentinio retinito gydymas
Maltese	Celluli alloġeniċi ġejjin mit-tessuti tal-kurdun taż-żokra uman	Kura tar-retinite pigmentuża
Polish	Allogeniczne ludzkie komórki pochodzące z tkanek pępowiny	Leczenie retinopatii barwnikowej
Portuguese	Células alogénicas derivadas de tecido do cordão umbilical humano	Tratamento de retinite pigmentosa
Romanian	Celule alogene provenite din cordon ombilical uman	Tratamentul retinitei pigmentare
Slovak	Alogénne bunky pochádzajúce z tkaniva ľudského pupočníka	Liečba retinitis pigmentosa
Slovenian	Alogenske celice, pridobljene iz humane popkovnične krvi	Zdravljenje pigmentozne retinopatije
Spanish	Células alogénicas obtenidas de tejido de cordón umbilical humano	Tratamiento de retinosis pigmentaria

[1] At the time of designation

Language	Active Ingredient	Indication
Swedish	Allogena celler från human navelsträngsvävnad	Behandling av retinitis pigmentosa
Norwegian	Celler fra allogent humant navlestrengsvev	Behandling av retinitis pigmentosa
Icelandic	Ósamgena mannafrumur úr naflastrengsvef	Meðferð áh retinitis pigmentosa