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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Expanded human allogeneic neural retinal progenitor cells extracted from neural retina for the treatment of retinitis pigmentosa

On 19 June 2013, orphan designation (EU/3/13/1140) was granted by the European Commission to ReNeuron Ltd, United Kingdom, for expanded human allogeneic neural retinal progenitor cells extracted from neural retina for the treatment of retinitis pigmentosa.

What is retinitis pigmentosa?

Retinitis pigmentosa is a group of hereditary diseases of the eye that lead to progressive loss of sight. In patients with retinitis pigmentosa, cells in the retina (the light-sensitive surface at the back of the eye) become damaged and eventually die.

Retinitis pigmentosa is a long-term debilitating disease because it causes the patient's sight to get worse, eventually leading to blindness.

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

At the time of designation, retinitis pigmentosa affected approximately 3.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 188,000 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 retinitis pigmentosa. Patients with the condition were given sunglasses to slow down the damage to the retina, genetic counselling (discussion of the risks of passing the condition on to children) and general support.

*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 27), Norway, Iceland and Liechtenstein. This represents a population of 509,000,000 (Eurostat 2013).

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How is this medicine expected to work?

The medicine contains retinal progenitor cells (or precursor cells) obtained from the eye and grown in a laboratory.

The medicine is intended for injection into the eye, under the retina. Once inside the retina, the cells in the medicine are expected to develop into mature retinal cells replacing the damaged cells. This is expected to help improve the person's sight.

What is the stage of development of this medicine?

The effects of the medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicinal product in patients with retinitis pigmentosa had been started.

At the time of submission, the medicinal product was not authorised anywhere in the EU for retinitis pigmentosa 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 15 May 2013 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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Expanded human allogeneic neural retinal progenitor cells extracted from neural retina	Treatment of retinitis pigmentosa
Bulgarian	Експанзирани човешки алогенни невринални ретинални прогенитор- клетки, извлечени от неврална ретина.	Лечение на пигментен ретинит
Czech	Expandované humánní allogenní progenitorové buňky neurální retiny extrahované z neurální retiny	Léčba pigmentosní retitinitidy
Danish	Ekspanderede humane allogene neurale retinale progenitor celler udtaget fra det neurale cellelag i retina	Behandling af retinitis pigmentosa
Dutch	Geëxpandeerde humane allogene neurale retinale progenitorcellen geëxtraheerd uit neurale retina	Behandeling van retinitis pigmentosa
Estonian	Neuraalreetinast ekstraheeritud laiendatud inimese allogeensed neuraalreetina progenitoorsed rakud	Pigmentoosse võrkkestapõletiku ravi
Finnish	Allogeeniset ihmisen neuraalisesta verkkokalvosta eristetyt ja kasvatetut progenitorisolut	Verkkokalvorappeuman hoito
French	Cellules souches neuro-rétiniennes allogéniques issues de cellules neuro-rétiniennes humaines amplifiées en culture	Traitement de la rétinite pigmentaire
German	Allogene, in-vitro vermehrte, neuronale Vorläuferzellen der Netzhaut, die aus der neuronalen Retina gewonnen wurden	Behandlung der Retinopathia Pigmentosa
Greek	Καλλιεργημένα ανθρώπινα αλλογενή νευρικά αμφιβληστορειδικά προγονικά κύτταρα απομονωμένα από αμφιβληστροειδή	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Neurális retinából kivont expandált humán allogén neurális retina progenitor sejtek	Retinitis pigmentosa kezelése
Italian	Cellule progenitrici allogeniche della retina neurale espanse, estratte dalla retina neurale	Trattamento della retinite pigmentosa
Latvian	No retinas neirālās daļas izdalītas un kultivētas cilvēka allogēnās neirālās retīnas priekšūnas	Retinitis pigmentosa ārstēšana
Lithuanian	Padaugintos alogeninės žmogaus tinklainės nervinio sluoksnio kamieninės ląstelės pirmtakės, gautos iš tinklainės nervinio sluoksnio	Pigmentinio retinito gydymas
Maltese	Ċelluli nevrali retinali alloġeneiċi proġenituri umani estratti mir-retina nevrali	Kura tar-retinite pigmentuża
Polish	Namnożone ludzkie allogeniczne komórki progenitorowe siatkówki uzyskane z siatkówki	Leczenie retinopatii barwnikowej

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	Células progenitoras de retina neural alogénicas humanas expandidas extraídas de retina neural	Tratamento da retinite pigmentosa
Romanian	Celule progenitoare neuronale retiniene umane alogene expandate extrase de la nivelul retinei neuronale	Tratamentul retinitei pigmentare
Slovak	Expandované ľudské alegénne bunky progenitora neurálnej retiny extrakované z neurálnej retiny	Liečba retinitis pigmentosa
Slovenian	Ekspandirane humane alogene nevralne mrežnične predniške celice, ekstrahirane iz nevralne mrežnice	Zdravljenje pigmentozne retinopatije
Spanish	Células progenitoras humanas alogénicas de retina neural expandidas extraidas de retina neural	Tratamiento de retinosis pigmentaria
Swedish	Expanderade humana allogena neurala retinala progenitorceller extraherade ur neural näthinna	Behandling av retinitis pigmentosa
Norwegian	Ekspanderte humane allogene neural retina progenitorceller ekstrahert fra neural retina	Behandling av retinitis pigmentosa
Icelandic	Útvíkkaðar manna tauga sjónu forstigsfrumur útdregnar úr tauga sjónu	Meðferð á retinitis pigmentosa