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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic fetal human retinal progenitor cells expanded ex vivo for the treatment of retinitis pigmentosa

On 17 February 2016, orphan designation (EU/3/16/1620) was granted by the European Commission to Voisin Consulting S.A.R.L., France, for allogeneic fetal human retinal progenitor cells expanded ex vivo 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 less than 3.7 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 190,000 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 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?

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

The medicine is intended for injection into the vitreous humour (the jelly-like fluid) of the eye. Once present in the eye, the progenitor cells are expected to produce proteins called neurotrophic factors which may encourage the growth of the patient's own retinal cells and help protect retinal cells from further damage. This is expected to slow the progress of the disease and improve the patient's ability to see.

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, clinical trials with this medicine in patients with retinitis pigmentosa were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for retinitis pigmentosa. Orphan designation of this medicine had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 January 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	Allogeneic fetal human retinal progenitor cells expanded ex vivo	Treatment of retinitis pigmentosa
Bulgarian	Алогенни човешки фетални ретинални прогениторни клетки, култивирани ex vivo	Лечение на пигментен ретинит
Croatian	Alogenične fetalne humane retinalne progenitorne stanice ekspandirane ex vivo	Liječenje retinitisa pigmentoze
Czech	Alogenní fetální lidské retinální progenitorové buňky expandované ex vivo	Léčba pigmentosní retinitidy
Danish	Allogene føtale humane retinale progenitorceller ekspanderet ex vivo	Behandling af retinitis pigmentosa
Dutch	Ex vivo geëxpandeerde allogene foetale humane retinale progenitorcellen	Behandeling van retinitis pigmentosa
Estonian	Allogeensed inimloote võrkkesta ex vivo kasvatatud eellasrakud	Pigmentoosse võrkkestapõletiku ravi
Finnish	Allogeeniset elimistön ulkopuolella monistetut ihmissikiön verkkokalvon esisolut	Verkkokalvorappeuman hoito
French	Cellules précurseurs rétiniennes fœtales humaines allogéniques amplifiées ex vivo	Traitement de la rétinite pigmentaire
German	Ex-vivo expandierte humane allogene fetale e Vorläufer Netzhautzellen	Behandlung der Retinopathia Pigmentosa
Greek	Αλλογενή εμβρυικά ανθρώπινα αμφιβληστροειδικά προγονικά κύτταρα πολλαπλασιασμένα ex vivo	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Ex vivo expandált humán allogén embrionális retina progenitor sejtek	Retinitis pigmentosa kezelése
Italian	Cellule progenitrici retiniche allogeniche fetali umane espanse ex vivo	Trattamento della retinite pigmentosa
Latvian	Ex vivo pavairotas alogēnas cilvēka augļa tīklenes progenitoru šūnas	*Retinitis pigmentosa* ārstēšana
Lithuanian	Alogeninės žmogaus embriono tinklainės pirmtakų ląstelės, pagausintos *ex vivo*	Pigmentinio retinito gydymas
Maltese	Ċelluli proġenituri retinali tal-fetu umani alloġeniċi mwassa' ex vivo	Kura tar-retinite pigmentuża
Polish	Allogeniczne, ludzkie, pozyskiwane z płodu komórki prekursorowe siatkówki ekspandowane ex vivo	Leczenie retinopatii barwnikowej
Portuguese	Células progenitoras retinianas alogénicas fetais humanas expandidas ex vivo	Tratamento da retinite pigmentosa
Romanian	Celule fetale progenitoare retiniene alogenice umane expandate ex vivo	Tratamentul retinitei pigmentare
Slovak	Alogénne fetálne ľudské retinálne progenitorové bunky expandované ex vivo	Liečba retinitis pigmentosa

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Alogene fetalne človeške mrežnične progenitorne celice, ekspandirane ex vivo	Zdravljenje pigmentozne retinopatije
Spanish	Células progenitoras retinianas fetales humanas alogénicas expandidas ex vivo	Tratamiento de la retinosis pigmentaria
Swedish	Ex-vivoexpanderade humana fetala retinala allogena progenitorceller	Behandling av retinitis pigmentosa
Norwegian	Allogene føtale progenitorceller fra humane retina, ekspandert ex vivo	Behandling av retinitis pigmentosa
Icelandic	Ósamgena stofnfrumur úr sjónhimnu fóstra ræktaðar ex vivo	Meðferð á retinitis pigmentosa