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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor for the treatment of retinitis pigmentosa

On 24 January 2013, orphan designation (EU/3/12/1098) was granted by the European Commission to Enpharma, United Kingdom, for encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor for 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 in 10,000 people in the European Union (EU). This was equivalent to a total of around 153,000 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).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

How is this medicine expected to work?

The medicine consists of human retinal cells that have been modified to contain a gene responsible for the production of a protein called ciliary neurotrophic factor (CNTF). The cells are contained in a small capsule that is surgically inserted into the eye. Once implanted into the eye, the retinal cells are expected to continuously produce and release CNTF, which stimulates and protects nerve cells such as specialised 'photoreceptor' cells responsible for detecting light in the retina of the eye. This is expected to prevent the loss of vision seen in retinitis pigmentosa.

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, clinical trials with the medicinal product in patients with retinitis pigmentosa were ongoing.

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 6 December 2012 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:

Enpharma Ltd
North House
Farmoor Court
Cumnor Road
Oxford, OX2 9LU
United Kingdom
Telephone: +44 199 3886 658; +1 401 33 338 80
Telefax: +1 401 33 338 81
E-mail: mail@enpharma.com

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	Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor	Treatment of retinitis pigmentosa
Bulgarian	Клетъчна линия от енкапсулирани човешки клетки от пигментен епител, трансфектирана с плазмиден вектор, експресиращ човешки цилиарен невротропен фактор.	Лечение на пигментен ретинит
Czech	Enkapsulovaná linie lidských epitelových pigmentových buněk sítnice transfekovaná plazmidovým vektorem exprimujícím lidský ciliární neurotrofický faktor	Léčba pigmentosní retitinitidy
Danish	Indkapslet human retinal pigment epithelial celle linje transfekteret med plasmid vektor udtrykkende human ciliær neurotrofisk faktor	Behandling af retinitis pigmentosa
Dutch	Geïncapsuleerd humaan retinaal pigment epitheliale cellijn getransfecteerd met plasmide vector dewelke humaan ciliair neurotrofische factor uitdrukt	Behandeling van retinitis pigmentosa
Estonian	Enkapsuleeritud inimese reetina pigmentepiteelirakud, millesse on viidud plasmiidi vektor, mis ekspresseerib inimese tsiliaarset neurotroopset faktorit.	Pigmentoosse võrkkestapõletiku ravi
Finnish	Kapseloitu ihmisen verkkokalvon pigmenttiepiteelisolulinja, jossa on ihmisen siliaarista neurotrofista tekijää ilmentävä plasmidivektori	Verkkokalvorappeuman hoito
French	Lignée cellulaire encapsulée de pigment retinien humain transfectée avec un vecteur plasmide expressant le facteur neurotrophique ciliaire humain	Traitement de la rétinite pigmentaire
German	Verkapselte, mit einem Plasmidvektor transfizierte humane retinale Pigmentepithel-Zelllinie, die humanen ziliären neurotrophischen Faktor exprimiert	Behandlung der Retinopathia Pigmentosa
Greek	Ενθυλακωμένη κυτταρική σειρά ανθώπινου μελαχρωστικού επιθηλίου διαμολυσμένου με ένα πλασμιδικό φορέα που εκφράζει τον ανθώπινο μικρολαχνιακό νευροτροφικό παράγοντα (CNTF)	Αγωγή κατά της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Kapszulába zárt plasmid vektorral transzferált humán ciliáris neurotrop faktort eszpresszáló humán retina epiteliális pigment sejtvonal	Retinitis pigmentosa kezelése
Italian	Linee cellulare encapsulate di pigmento retinale umano transfettate con un vettore plasmidico esprimente il fattore neurotrofico ciliare umano.	Trattamento della retinite pigmentosa
Latvian	Iekapsulētas cilvēka tīklenes pigmenta epiteliālo šūnu līnija, kurai veikta transfekcija ar plazmīdas faktoru, kurš ekspresē cilvēka ciliāro neirotrofisko faktoru	Retinitis pigmentosa ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Inkapsuliuota žmogaus tinklainės pigmentinio epitelio ląstelių linija, transfektuota su plazmidžių vektoriumi, ekspresuojančiu žmogaus ciliarinį neurotrofinį faktorių	Pigmentinio retinito gydymas
Maltese	Linja ta' ċelluli epiteljali umani ta' pigment retinali inkapsulati, transfettati b'vettur ta' plasmid li jesprimi l-fattur newrotrofiku ċiljari uman	Kura tar-retinite pigmentuża
Polish	Komórki linii siatkówki ludzkiej transfekowane wektorem plazmidowym wykazującym ekspresję ludzkiego rzęskowego czynnika neurotroficznego	Leczenie retinopatii barwnikowej
Portuguese	Linha de células do epitélio pigmentar da retina humana encapsuladas e transfectadas com um vector plasmídeo expressando o fator neurotrófico ciliar humano	Tratamento da retinite pigmentosa
Romanian	Pigment retinian uman encapsulat continut in linie celulara epiteliala transfectata cu vector plasmidial exprimand factor neutrotrofic ciliar uman	Tratamentul retinitei pigmentare
Slovak	Bunková línia epiteliálnych buniek s enkapsulovaným sietnicovým pigmentom transfektovaná plazmidovým vektorom exprimujúcim ľudský ciliárny neurotrofický faktor	Liečba retinitis pigmentosa
Slovenian	Inkapsulirana linija pigmentnih epitelnih celic , transficiranih s plazmidnim vektorjem za humani ciliarni nevrotrofni faktor	Zdravljenje pigmentozne retinopatije
Spanish	Linea celular humana del epitelio pigmentario retinal encapsulada transfectada con un vector plasmidico que codifica para factor neurotropico ciliar humano	Tratamiento de retinosis pigmentaria
Swedish	Inkapslad human retinalpigment-epitelcellslinje transfekterad med en plasmidvektor som uttrycker human ciliary neurotrophic factor	Behandling av retinitis pigmentosa
Norwegian	Innkapslet human retinal pigment epitelcellelinje transfektert med plasmid vektor som uttrykker human ciliær neurotrofisk faktor	Behandling av retinitis pigmentosa
Icelandic	Hylkisbundið manna sjónhimnu litarefnis epithelial frumulína sem er transfected með plasmíð ferju sem tjáir manna bifhára neurótróp faktor	Meðferð á retinitis pigmentosa