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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 neurotropic factor for the treatment of macular telangiectasia type 2

On 8 November 2012, orphan designation (EU/3/12/1072) was granted by the European Commission to Enpharma Ltd, United Kingdom, for encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotropic factor for the treatment of macular telangiectasia type 2.

What is macular telangiectasia type 2?

Macular telangiectasia type 2 is a disease that leads to the progressive loss of sight in both eyes. The disease usually does not cause symptoms in its earliest stage and is often not diagnosed until the fifth or sixth decades of life, when the patient begins to experience loss of vision. Although the origin of the disease is unknown, the disease is characterised by damage in the nerve cells in the macula (the central part of the retina, the light-sensitive surface at the back of the eye).

Macular telangiectasia type 2 is a long-term debilitating disease because it causes loss of vision.

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

At the time of designation, macular telangiectasia type 2 affected less than 2.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 116,000 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?

No satisfactory methods of treatment were authorised in the EU at the time of orphan designation.

*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 506,300,000 (Eurostat 2011).

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How is this medicine expected to work?

The medicine consists of human retinal cells that have been genetically modified to produce a human growth factor called ciliairy 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 macular telangiectasia type 2.

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 macular telangiectasia type 2 were ongoing.

At the time of submission, the medicinal product was not authorised anywhere in the EU for macular telangiectasia type 2. Orphan designation has been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 October 2012 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 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	Encapsulated human retinal pigment epithelial cell line transfected with plasmid vector expressing human ciliary neurotrophic factor	Treatment of macular telangiectasia type 2
Bulgarian	Клетъчна линия от енкапсулирани човешки клетки от пигментен епител, трансфектирана с плазмиден вектор, експресиращ човешки цилиарен невротропен фактор.	Лечение на макуларна телангиектазия тип 2.
Czech	Enkapsulovaná linie lidských epitelových pigmentových buněk sítnice transfekovaná plazmidovým vektorem exprimujícím lidský ciliární neurotrofický faktor	Léčba makulární teleangiektázie typu 2
Danish	Indkapslet human retinal pigment epithelial celle linje transfekteret med plasmid vektor udtrykkende human ciliær neurotrofisk faktor	Behandling af maculær telangiectasi type 2
Dutch	Geïncapsuleerd humaan retinaal pigment epitheliale cellijn getransfecteerd met plasmide vector dewelke humaan ciliair neurotrofische factor uitdrukt	Behandeling van maculaire telangiëctasie type 2
Estonian	Enkapsuleeritud inimese reetina pigmentepiteelirakud, millesse on viidud plasmiidi vektor, mis ekspresseerib inimese tsiliaarset neurotroopset faktorit.	2. tüüpi makulaarse teleangiektaasia ravi
Finnish	Kapseloitu ihmisen verkkokalvon pigmenttiepiteelisolulinja, jossa on ihmisen siliaarista neurotrofista tekijää ilmentävä plasmidivektori	Tyypin 2 makulaarisen telangiektasian hoito
French	Lignée cellulaire encapsulée de pigment retinien humain transfectée avec un vecteur plasmide expressant le facteur neurotrophique ciliaire humain	Traitement de la telangiectasia maculaire de type 2
German	Verkapselte, mit einem Plasmidvektor transfizierte humane retinale Pigmentepithel-Zelllinie, die humanen ziliären neurotrophischen Faktor exprimiert	Behandlung der makulären Teleangiektasie Typ 2
Greek	Ενθυλακωμένη κυτταρική σειρά ανθώπινου μελαχρωστικού επιθηλίου διαμολυσμένου με ένα πλασμιδικό φορέα που εκφράζει τον ανθώπινο μικρολαχνιακό νευροτροφικό παράγοντα (CNTF)	Θεραπεία της τηλαγγειεκτασίας της ωχράς κηλίδας τύπου 2
Hungarian	Kapszulába zárt plasmid vektorral transzferált humán ciliáris neurotrop faktort eszpresszáló humán retina epiteliális pigment sejtvonal	2-típusú maculáris telangiectasia kezelése
Italian	Linee cellular encapsulate di pigmento retinale umano transfettate con un vettore plasmidico esprimente il fattore neurotrofico ciliare umano.	Trattamento della teleangectasia maculare di tipo 2
Latvian	Iekapsulētas cilvēka tīklenes pigmenta epiteliālo šūnu līnija, kurai veikta transfekcija ar plazmīdas	2 tipa makulārās teleangektāzijas ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
	faktoru, kurš ekspresē cilvēka ciliāro neirotrofisko faktoru	
Lithuanian	Inkapsuliuota žmogaus tinklainės pigmentinio epitelio ląstelių linija transfektuota su plazmidžių vektoriumi, ekspresuojančiu žmogaus ciliarinį neurotrofinį	II tipo makulinės telangiektazijos gydymas
Maltese	Linja ta' ċelluli epiteljali umani ta' pigment retinali inkapsulati, transfettati b'vettur ta' plasmid li jesprimi l-fattur newrotrofiku ċiljari uman	Kura tat-telanġektasija makulari tat-tip 2
Polish	Komórki linii siatkówki ludzkiej transfekowane wektorem plazmidowym wykazującym ekspresję ludzkiego rzęskowego czynnika neurotroficznego	Leczenie teleangiektazji typu 2 plamki żółtej
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 telangiectasia macular tipo 2
Romanian	Pigment retinian uman encapsulat continut in linie celulara epiteliala transfectata cu vector plasmidial exprimand factor neutrotrofic ciliar uman	Tratamentul teleangectaziei maculare de tip 2
Slovak	Bunková línia epiteliálnych buniek s enkapsulovaným sietnicovým pigmentom transfektovaná plazmidovým vektorom exprimujúcim ľudský ciliárny neurotrofický faktor	Liečba makulárnej teleangiektázie typ 2
Slovenian	Inkapsulirana linija pigmentnih epitelnih celic , transficiranih s plazmidnim vektorjem za humani ciliarni nevrotrofni faktor	Zdravljenje makularne telangiektazije tipa 2
Spanish	Linea celular humana del epitelio pigmentario retinal encapsulada transfectada con un vector plasmidico que codifica para factor neurotropico ciliar humano	Tratamiento de la telangiectasia macular de tipo 2
Swedish	Inkapslad human retinalpigment-epitelcellslinje transfekterad med en plasmidvektor som uttrycker human ciliary neurotrophic factor	Behandling av makulär telangiektasi typ 2
Norwegian	Innkapslet human retinal pigment epitelcellelinje transfektert med plasmid vektor som uttrykker human ciliar neurotrop faktor	Behandling av makulær telangiektasi type 2
Icelandic	Hylkisbundið manna sjónhimnu litarefnis epithelial frumulína sem er transfected með plasmíð ferju sem tjáir manna bifhára neurótróp faktor	Meðferð augnbotna telangíéktasíu gerð 2