25 February 2015
EMA/COMP/473374/2007 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human rod-derived cone viability factor for the treatment of retinitis pigmentosa

First publication	1 July 2008
Rev.1: sponsor's change of address	20 August 2009
Rev.2: withdrawal from the Community Register	25 February 2015

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2014 on request of the Sponsor.

On 29 November 2007, orphan designation (EU/3/07/500) was granted by the European Commission to Fovea Pharmaceuticals SA, France, for recombinant human rod-derived cone viability factor for the treatment of 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 less than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 150,000 people*, and is below the ceiling for

*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.
At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

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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.

How is this medicine expected to work?

Recombinant human rod-derived cone viability factor (rh-RdCVF) is a protein, composed of 109 blocks called amino acids. This protein is normally produced by some cells of the retina, called rods. The product is expected to protect other cells in the retina, called cones, from being damaged. By administering additional quantities of this substance directly inside the eye, it is expected that vision will be preserved for a longer time.

What is the stage of development of this medicine?

The effects of recombinant human rod-derived cone viability factor were evaluated in experimental models.

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

The medicinal product was not authorised anywhere in the world for retinitis pigmentosa or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 October 2007 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	Recombinant human rod derived cone viability factor	Treatment of retinitis pigmentosa
Bulgarian	Рекомбинантни пръчици,получени от жизнеспособен фактор на човешки колбички	Лечение на пигментен ретинит
Czech	Rekombinantní lidský faktor životnosti čípků odvozený z tyčinek	Léčba pigmentosní retitinitidy
Danish	Rekombinant human stavderiveret tapviabilitetsfaktor	Behandling af retinitis pigmentosa
Dutch	Humaan recombinant staafafgeleide kegelviabiliteitsfactor	Behandeling van retinitis pigmentosa
Estonian	Rekombinantne inimese kepikestest pärinev kolvikeste elujõulisuse faktor	Pigmentoosse võrkkestapõletiku ravi.
Finnish	Yhdistelmä-DNA-tekniikalla valmistettu ihmisen sauvasolusta johdettu tappisolun elinkykytekijä (RdCVF)	Hoito verkkokalvorappeumaan
French	Facteur humain recombinant dérivé des bâtonnets pour la viabilité des cônes	Traitement de la rétinite pigmentaire
German	Von Stäbchen stammender rekombinanter humaner Überlebensfaktor für Zapfen	Behandlung der Retinopathia Pigmentosa
Greek	Ανασυνδυασμένος ανθρώπινος προερχόμενος από τα ραβδία παράγων βιωσιμότητας των κωνίων	Αγωγή κατά της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Rekombináns humán csapsejtből származó pálcika viabilitási faktor	Retinitis pigmentosa kezelése
Italian	Fattore di vitalità dei coni prodotto dai bastoncelli umani, ricombinante	Trattamento della retinite pigmentosa
Latvian	Rekombinētās cilvēka tīklenes nūjiņās atrodams par fotoreceptoru šūnu dzīvotspēju atbildīgs gēns	Retinitis pigmentosa ārstēšana
Lithuanian	Rekombinantinis žmogaus lazdelių gyvybingumo faktorius gautas iš kolbelių	Pigmentinio retinito gydymas
Maltese	Fattur ta' vijabilità tal-kon imnissel mill-vireg umani rikombinanti	Kura tar-retinite pigmentuża
Polish	Rekombinant ludzkiego czynnika żywotności czopków uzyskanego z pręcików	Leczenie retinopatii barwnikowej
Portuguese	Factor recombinante humano de viabilidade dos cones derivado dos bastonetes	Tratamento de retinite pigmentosa
Romanian	Factor recombinant uman de viabilitate a conurilor, provenit din bastonaşe retiniene (Rod-derived Cone Viability Factor= RdCVF)	Tratamentul retinitei pigmentare

[1] At the time of designation

Language	Active Ingredient	Indication
Slovak	Rekombinantný, ľudský, z tyčiniek získaný faktor životaschopnosti čapíkov	Liečba retinitis pigmentosa
Slovenian	Rekombinantni humani faktor viabilnosti čepnic, pridobljen iz paličnic	Zdravljenje pigmentozne retinopatije
Spanish	Factor recombinante humano de viabilidad de los conos derivado de los bastones	Tratamiento de retinosis pigmentaria
Swedish	Rekombinant human stavderiverad tappviabilitetsfaktor	Behandling av retinitis pigmentosa
Norwegian	Rekombinant human stav-derivert tappviabilitetsfaktor	Behandling av retinitis pigmentosa
Icelandic	Raðbrigða sjónkeilulífefni unnið úr stafþekjufrumum úr mönnum	Meðferð áh retinitis pigmentosa