



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human methionine proinsulin for the treatment of retinitis pigmentosa

On 26 April 2012, orphan designation (EU/3/12/985) was granted by the European Commission to ProRetina Therapeutics S.L., Spain, for recombinant human methionine proinsulin 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 this condition?

At the time of designation, retinitis pigmentosa affected less than 3.7 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 187,000 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 506,300,000 (Eurostat 2011).



How is this medicine expected to work?

This medicine contains the protein proinsulin, which in the body is normally converted to insulin, the hormone that controls the levels of glucose (sugar) in blood. When injected into the eye of patients with retinitis pigmentosa, proinsulin is thought to reduce the death of retina cells. This is expected to slow down and prevent the loss of retina cells in these patients.

The medicine is produced by a method known as 'recombinant DNA technology': it is made by bacteria that have received a gene (DNA), which makes them able to produce proinsulin.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with retinitis pigmentosa had been started.

At the time of submission, recombinant human methionine proinsulin 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 8 March 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant human methionine proinsulin	Treatment of retinitis pigmentosa
Bulgarian	Рекомбинантен човешки метионин проинсулин	Лечение на пигментен ретинит
Czech	Rekombinantní lidský methionin proinsulin	Léčba pigmentosní retinitidy
Danish	Rekombinant human methionin proinsulin	Behandling af retinitis pigmentosa
Dutch	Recombinant humaan methionine pro-insuline	Behandeling van retinitis pigmentosa
Estonian	Inimese rekombinantne metioniin-proinsuliin	Pigmentoosse võrkkestapõletiku ravi
Finnish	Rekombinantti ihmisen proinsuliini	Verkkokalvorappeuman hoito
French	Proinsuline méthionine recombinante humaine	Traitement de la rétinite pigmentaire
German	Rekombinantes humanes Methionin-Proinsulin	Behandlung der Retinopathia Pigmentosa
Greek	Ανασυνδυασμένη ανθρώπινη μεθειονίνη-προινσουλίνη	Αγωγή κατά της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Rekombináns humán methionin-proinsulin	Retinitis pigmentosa kezelése
Italian	Proinsulina metionina umana ricombinante	Trattamento della retinite pigmentosa
Latvian	Rekombinants cilvēka Proinsulīns	Retinitis pigmentosa ārstēšana
Lithuanian	Žmogaus rekombinantinis metionino proinsulinas	Pigmentinio retinito gydymas
Maltese	Proinsulina methionine umana rikombinanti	Kura tar-retinite pigmentuża
Polish	Rekombinowana ludzka proinsulina metioninowa	Leczenie retinopatii barwnikowej
Portuguese	Proinsulina Metionina humana recombinante	Tratamento da retinite pigmentosa
Romanian	Metionin-proinsulină umană recombinantă	Tratamentul retinitei pigmentare
Slovak	Rekombinantný ľudský metionín proinzulín	Liečba retinitis pigmentosa
Slovenian	Rekombinantni humani metionin proinsulin	Zdravljenje pigmentozne retinopatije
Spanish	Proinsulina metionina recombinante humana	Tratamiento de retinosis pigmentaria
Swedish	Rekombinant humant metionin proinsulin	Behandling av retinitis pigmentosa
Norwegian	Rekombinant human metionin proinsulin	Behandling av retinitis pigmentosa
Icelandic	Raðbrigða manna methiólnín forinsúlín	Meðferð á retinitis pigmentosa

¹ At the time of designation