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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

recombinant human proinsulin for the treatment of retinitis pigmentosa

On 11 February 2009, orphan designation (EU/3/08/612) was granted by the European Commission to ProRetina Therapeutics S.L., Spain, for recombinant human proinsulin for the treatment of retinitis pigmentosa.

What is retinitis pigmentosa?

Retinitis pigmentosa is a genetic (hereditary) disease of the eye that leads to progressive loss of sight. In patients with retinitis pigmentosa, some 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 the patient's sight becomes progressively worse and eventually leads to blindness.

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

At the time of designation retinitis pigmentosa affected approximately 2.9 in 10,000 people in the European Union (EU)*. This is below the threshold for orphan designation, which is 5 in 10,000, and is equivalent to a total of around 145,700 people. This is based on the information provided by the sponsor and 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 methods were authorised in the EU for treating the condition. Patients with retinitis pigmentosa were given genetic counselling (discussion of the risk of passing the condition on to children), and general support such as information and regular medical follow-up.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).

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How is this medicine expected to work?

Proinsulin is a precursor of insulin. This means that recombinant human proinsulin is converted into insulin in the body. Insulin is the hormone that regulates blood sugar levels. However, it can also block cell death. When injected directly into the eyes of patients with retinitis pigmentosa, recombinant human proinsulin is expected to help reduce cell death in the retina, slowing down or preventing the loss of sight.

Recombinant human proinsulin is produced by a method known as 'recombinant DNA technology': it is made by a bacterium that has received a gene in the form of DNA which makes it able to produce human proinsulin.

What is the stage of development of this medicine?

The effects of recombinant human proinsulin have been 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 started.

At the time of submission, recombinant human proinsulin was not authorised anywhere in the world 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 10 December 2008 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 European Union) 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

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Recombinant human proinsulin	Treatment of retinitis pigmentosa
Bulgarian	Рекомбинантен човешки проинсулин	Лечение на пигментен ретинит
Czech	Rekombinantní lidský proinsulin	Léčba pigmentosní retitinitidy
Danish	Rekombinant humant proinsulin	Behandling af retinitis pigmentosa
Dutch	Recombinant humaan proinsulin	Behandeling van retinitis pigmentosa
Estonian	Inimese rekombinantne proinsuliin	Pigmentoosse võrkkestapõletiku ravi
Finnish	Rekombinantti ihmisen proinsuliini	Verkkokalvorappeuman hoito
French	Proinsuline humaine recombinante	Traitement de la rétinite pigmentaire
German	Rekombinanter humaner Proinsulin	Behandlung der Retinopathia Pigmentosa
Greek	Ανασυνδυασμένος ανθρώπινος προινσουλίνη	Αγωγή κατά της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Rekombináns humán proinsulin	Retinitis pigmentosa kezelése
Italian	Proinsulina umana ricombinante	Trattamento della retinite pigmentosa
Latvian	Rekombinants cilvēka proinsulīns	Retinitis pigmentosa ārstēšana
Lithuanian	Rekombinantinis žmogaus proinsulinas	Pigmentinio retinito gydymas
Maltese	Proinsulina rikombinanti umana	Kura tar-retinite pigmentuża
Polish	Rekombinowana ludzka proinsulina	Leczenie retinopatii barwnikowej
Portuguese	Proinsulina humano recombinante	Tratamento da retinite pigmentosa
Romanian	Proinsulină umană recombinantă	Tratamentul retinitei pigmentare
Slovak	Rekombinantný ľudský proinzulín	Liečba retinitis pigmentosa
Slovenian	Rekombinantni humani proinsulin	Zdravljenje pigmentozne retinopatije
Spanish	Proinsulina humana recombinante	Tratamiento de retinosis pigmentaria
Swedish	Rekombinant human proinsulin	Behandling av retinitis pigmentosa
Norwegian	Rekombinant human proinsulin	Behandling av retinitis pigmentosa
Icelandic	Raðbrigða manna forinsúlín	Meðferð á retinitis pigmentosa

[1] At the time of designation