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EMA/COMP/167703/2011 Rev 2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

9-*cis*-Retinyl acetate for the treatment of retinitis pigmentosa

On 13 May 2011, orphan designation (EU/3/11/865) was granted by the European Commission to ORS Oxford Ltd, United Kingdom, for 9-*cis*-retinyl acetate for the treatment of retinitis pigmentosa. The sponsorship was transferred to QLT Ophthalmics (UK), Ltd, United Kingdom, in November 2011.

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 was estimated to affect less than 3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 152,000 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 submission of the application for orphan 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?

11-*cis*-Retinal is a substance derived from vitamin A that the specialised light-sensitive photoreceptor cells in the retina need, to be able to translate light into nerve impulses. In retinitis pigmentosa, the retina does not produce or regenerate enough of 11-*cis*-retinal and the photoreceptor cells become depleted and damaged.

9-*cis*-Retinyl acetate is expected to be taken by mouth and converted into 9-*cis*-retinal in the retina. 9-*cis*-Retinal is similar to the deficient 11-*cis*-retinal and is expected to replace it, thus enabling the photoreceptor cells to function properly.

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 9-*cis*-retinyl acetate in experimental models was ongoing.

At the time of submission, clinical trials with 9-*cis*-retinyl acetate in patients with retinitis pigmentosa were ongoing.

At the time of submission, 9-*cis*-retinyl acetate 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 9 February 2011 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:

QLT Ophthalmics (UK), Ltd
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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	9- <i>cis</i> -Retinyl acetate	Treatment of retinitis pigmentosa
Bulgarian	9-цис-ретинил ацетат	Лечение на пигментен ретинит
Czech	9- <i>cis</i> -retinylacetát	Léčba pigmentosní retinitidy
Danish	9- <i>cis</i> -retinylacetat	Behandling af retinitis pigmentosa
Dutch	9- <i>cis</i> -retinylacetaat	Behandeling van retinitis pigmentosa
Estonian	9- <i>cis</i> -retinüülatsetaat	Pigmentoosse võrkkestapõletiku ravi
Finnish	9- <i>cis</i> -retinyyliasetatti	Verkkokalvorappeuman hoito
French	9 <i>cis</i> rétinyl acetate	Traitement de la rétinite pigmentaire
German	9- <i>cis</i> -Retinylacetat	Behandlung der Retinopathia Pigmentosa
Greek	9- <i>cis</i> -οξεική ρετινόλη	Αγωγή κατά της μεταγγρωστικής αμφιβληστροειδοπάθειας
Hungarian	9- <i>cis</i> z-retinil-acetát	Retinitis pigmentosa kezelése
Italian	9- <i>cis</i> -retinil acetato	Trattamento della retinite pigmentosa
Latvian	9- <i>cis</i> -retinilacetāts	Retinitis pigmentosa ārstēšana
Lithuanian	9- <i>cis</i> -retinilacetatas	Pigmentinio retinito gydymas
Maltese	9- <i>cis</i> -retinyl acetate	Kura tar-retinite pigmentuża
Polish	Octan 9- <i>cis</i> -retinyłu	Leczenie retinopatii barwnikowej
Portuguese	Acetato de 9- <i>cis</i> -retinil	Tratamento da retinite pigmentosa
Romanian	9- <i>cis</i> -retinilacetat	Tratamentul retinitei pigmentare
Slovak	9- <i>cis</i> -retinylacetát	Liečba retinitis pigmentosa
Slovenian	9- <i>cis</i> -retinil acetat	Zdravljenje pigmentozne retinopatije
Spanish	9- <i>cis</i> -retinil acetato	Tratamiento de retinosis pigmentaria
Swedish	9- <i>cis</i> -retinylacetat	Behandling av retinitis pigmentosa
Norwegian	9- <i>cis</i> -retinylacetat	Behandling av retinitis pigmentosa
Icelandic	9- <i>cis</i> -retinýlasetat	Meðferð á retinitis pigmentosa

¹ At the time of designation