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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human nerve growth factor for the treatment of retinitis pigmentosa

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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.	

On 7 June 2013, orphan designation (EU/3/13/1135) was granted by the European Commission to Dompé S.p.A., Italy, for recombinant human nerve growth factor for the treatment of retinitis pigmentosa.

The sponsorship was transferred to Dompé farmaceutici s.p.a., Italy, in March 2015.

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 affected approximately 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 154,000 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).

*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 28), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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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 genetic counselling (discussion of the risks of passing the condition on to children) and general support.

How is this medicine expected to work?

Human nerve growth factor (NGF) is a protein produced in the body that plays an important role in the growth and survival of nerve cells, including the cells of the retina. Patients with retinitis pigmentosa have lower than normal levels of NGF in the retina. Recombinant human NGF given as eye drops to patients with retinitis pigmentosa is expected to replace the missing NGF and improve the survival of retina cells, thereby slowing the development of the disease and helping to preserve vision.

The medicine is produced by a method known as 'recombinant DNA technology': it is made by bacteria into which a gene (DNA) has been introduced that makes them able to produce NGF.

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 recombinant human nerve growth factor in experimental models was ongoing, but no clinical trials with the medicine in patients with retinitis pigmentosa had been started.

At the time of submission, the medicine 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 17 April 2013 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 nerve growth factor	Treatment of retinitis pigmentosa
Bulgarian	Рекомбинантен човешки неврален растежен фактор	Лечение на пигментен ретинит
Croatian	Rekombinantni ljudski faktor rasta živaca	Liječenje retinitisa pigmentoze
Czech	Rekombinantní lidský nervový růstový faktor	Léčba pigmentosní retitinitidy
Danish	Rekombinant human nerve vækstfaktor	Behandling af retinitis pigmentosa
Dutch	Recombinant humaan zenuwweefsel groeifactor	Behandeling van retinitis pigmentosa
Estonian	Rekombinantne inimese närvi kasvufaktor	Pigmentoosse võrkkestapõletiku ravi
Finnish	Rekombinantti ihmisen hermokasvutekijä	Verkkokalvorappeuman hoito
French	Facteur de croissance recombinant du tissu nerveux humain	Traitement de la rétinite pigmentaire
German	Rekombinanter humaner Nervenwachstumsfaktor	Behandlung der Retinopathia Pigmentosa
Greek	Ανασυνδυασμένος ανθρώπινος αυξητικός παράγοντας νεύρων	Αγωγή κατά της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Rekombináns humán idegsejt-növekedési faktor	Retinitis pigmentosa kezelése
Italian	Fattore di crescita del tessuto nervoso ricombinante umano	Trattamento della retinite pigmentosa
Latvian	Rekombinēts cilvēka nervu augšanas faktors	Retinitis pigmentosa ārstēšana
Lithuanian	Rekombinantinis žmogaus nervų augimo faktorius	Pigmentinio retinito gydymas
Maltese	Fattur tat-tkabbir tan-nervituri rikombinanti uman	Kura tar-retinite pigmentuża
Polish	Rekombinowany ludzki czynnik wzrostu nerwu	Leczenie retinopatii barwnikowej
Portuguese	Fator de crescimento neural humano recombinante	Tratamento da retinite pigmentosa
Romanian	Factor recombinant uman de creştere a ţesutului nervos	Tratamentul retinitei pigmentare
Slovak	Rekombinantný ľudský nervový rastový faktor	Liečba retinitis pigmentosa
Slovenian	Rekombinantni humani živčni rastni dejavnik	Zdravljenje pigmentozne retinopatije

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Factor de crecimiento del nervio humano recombinante	Tratamiento de retinosis pigmentaria
Swedish	Rekombinant mänsklig nervtillväxtfaktor	Behandling av retinitis pigmentosa
Norwegian	Rekombinant human nervevekstfaktor	Behandling av retinitis pigmentosa
Icelandic	Raðbrigða manna taugavaxtarþáttur	Meðferð á retinitis pigmentosa