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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 neurotrophic keratitis

On 14 December 2015, orphan designation (EU/3/15/1586) was granted by the European Commission to Dompé farmaceutici S.p.A., Italy, for recombinant human nerve growth factor for the treatment of neurotrophic keratitis.

What is neurotrophic keratitis?

Neurotrophic keratitis is a condition of the cornea (the clear layer at the front of the eye) caused by damage to the trigeminal nerve, the nerve that allows the surface of the eye to feel things and which supplies growth factors and other substances needed for normal growth and repair of the eye's surface. The nerve damage results in a lack of sensitivity in the cornea, and to dryness, ulceration and scarring that interferes with vision.

Neurotrophic keratitis is a long-lasting and debilitating condition due to damage to the cornea and possible loss of sight.

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

At the time of designation, neurotrophic keratitis affected approximately 4.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 215,000 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?

No satisfactory methods were authorised for the treatment of neurotrophic keratitis in the EU at the time of designation. Management depended on the stage of the disease and included supportive measures such as eye drops to moisten and lubricate the eye, antibiotics for eye infections, and the use of protective contact lenses, as well as surgery where appropriate.

*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. This represents a population of 512,900,000 (Eurostat 2015).

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How is this medicine expected to work?

Patients with neurotrophic keratitis have lower than normal levels of growth factors that are normally supplied by the trigeminal nerve and which play an important role in the growth and survival of the cells of the cornea. The medicine is a copy of a human growth factor called nerve growth factor. When given as eye drops to patients with neurotrophic keratitis, the medicine is expected to help restore some of the normal healing processes in the eye and repair the damages to the cornea associated with the condition.

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 enables the bacteria to produce human nerve growth factor.

What is the stage of development of this medicine?

The effects of recombinant human nerve growth factor have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with recombinant human nerve growth factor in patients with neurotrophic keratitis were ongoing.

At the time of submission, recombinant human nerve growth factor was not authorised anywhere in the EU for neurotrophic keratitis. Orphan designation of the medicine had been granted in the United States for neurotrophic keratitis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 November 2015 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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 neurotrophic keratitis
Bulgarian	Рекомбинантен човешки неврален растежен фактор	Лечение на невротрофичен кератит
Croatian	Rekombinantni ljudski faktor rasta živaca	Liječenje neurotrofnog keratitisa
Czech	Rekombinantní lidský nervový růstový faktor	Léčba neurotrofické keratitidy
Danish	Rekombinant human nerve vækstfaktor	Behandling af neurotrofisk keratitis
Dutch	Recombinant humaan zenuwweefsel groeifactor	Behandeling van neurotrofische keratitis
Estonian	Rekombinantne inimese närvikasvufaktor	Neurotroofilise keratiidi ravi
Finnish	Rekombinantti ihmisen hermokasvutekijä	Neurotrofisen keratiitin hoito
French	Facteur de croissance recombinant du tissu nerveux humain	Traitement de la kératite neurotrophique
German	Rekombinanter humaner Nervenwachstumsfaktor	Behandlung der Neurotrophen Keratitis
Greek	Ανασυνδυασμένος ανθρώπινος αυξητικός παράγοντας νεύρων	θεραπεία της νευροτροφικής κερατίτιδας
Hungarian	Rekombináns humán idegsejt-növekedési faktor	Neurotrofiás keratitis kezelése
Italian	Fattore di crescita del tessuto nervoso ricombinante umano	Trattamento della cheratite neurotrofica
Latvian	Rekombinēts cilvēka nervu augšanas faktors	Neirotropā keratīta ārstēšana
Lithuanian	Rekombinantinis žmogaus nervų augimo faktorius	Neurotrofinio keratito gydymas
Maltese	Fattur tat-tkabbir tan-nervituri rikombinanti uman	Kura tal-keratite newrotrofika
Polish	Rekombinowany ludzki czynnik wzrostu nerwu	Leczenie neurotroficznego zapalenia rogówki
Portuguese	Fator de crescimento neural humano recombinante	Tratamento da queratite neurotrófica
Romanian	Factor recombinant uman de creştere a ţesutului nervos	Tratamentul keratitei neurotrofice
Slovak	Rekombinantný ľudský nervový rastový faktor	Liečba neurotrofickej keratitídy
Slovenian	Rekombinantni humani živčni rastni dejavnik	Zdravljenje nevrotrofičnega keratitisa
Spanish	Factor de crecimiento del nervio humano recombinante	Tratamiento de la queratopatía neurotrófica
Swedish	Rekombinant mänsklig nervtillväxtfaktor	Behandling vid neurotrofisk keratit
Norwegian	Rekombinant human nervevekstfaktor	Behandling av nevrotrofisk keratitt
Icelandic	Raðbrigða manna taugavaxtarþáttur	Meðferð við taugarýrnunar glærubólga

[1] At the time of designation