19 May 2015
EMA/COMP/209499/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human mesencephalic astrocyte-derived neurotrophic factor for the treatment of retinitis pigmentosa

On 24 April 2015, orphan designation (EU/3/15/1486) was granted by the European Commission to Clinipace GmbH, Germany, for recombinant human mesencephalic astrocyte-derived neurotrophic factor 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 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).

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 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?

It is thought that in patients with retinitis pigmentosa the death of retinal cells may be linked to problems with a structure inside the cells called the endoplasmic reticulum, which is involved in the production of proteins and helps them to fold properly. When retinal cells are stressed in patients with the condition, proteins may misfold and accumulate, resulting in the death of the cells.

The medicine consists of a human protein called 'mesencephalic astrocyte-derived neurotrophic factor' (MANF) which is thought to be normally produced in response to stress and to assist with the correct folding of proteins. By reducing the build-up of misfolded proteins under stress, MANF is expected to help retinal cells to survive.

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

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 of the application for orphan designation, 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. Orphan designation had been granted for this medicine in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 March 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:

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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 mesencephalic astrocyte-derived neurotrophic factor	Treatment of retinitis pigmentosa
Bulgarian	Човешки рекомбинантен мезенцефален астроцитен невротрофичен фактор	Лечение на пигментен ретинит
Croatian	Rekombinantni ljudski neurotrofni faktor dobiven iz astrocita mezencefalona	Liječenje retinitisa pigmentoze
Czech	Lidský rekombinantní mezencefalický neurotrofický faktor odvozený od astrocytů	Léčba pigmentosní retitinitidy
Danish	Human rekombinant mesencephal astrocyt-deriveret neurotrofisk faktor	Behandling af retinitis pigmentosa
Dutch	Humaan recombinant mesencefaal astrocyt-afgeleide neurotrofe factor	Behandeling van retinitis pigmentosa
Estonian	Inimese rekombinantne keskaju astrotsüütidest pärinev neurotroofiline faktor	Pigmentoosse võrkkestapõletiku ravi
Finnish	Ihmisen rekombinantti mesenkefaalinen astrosyyttiperäinen hermokasvutekijä	Verkkokalvorappeuman hoito
French	Facteur humain recombinant neurotrophique dérivé des astrocytes mésencéphaliques	Traitement de la rétinite pigmentaire
German	Aus Astrozyten stammender humaner rekombinanter mesenzephalischer neurotropher Faktor	Behandlung der Retinopathia Pigmentosa
Greek	Ανασυνδυασμένος ανθρώπινος μεσεγκεφαλικός νευροτροφικός παράγοντας προερχόμενος από αστροκύτταρα	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Rekombináns humán középagyi asztrocita-eredetű neurotrofikus faktor	Retinitis pigmentosa kezelése
Italian	Fattore neurotrofico ricombinante umano derivato dagli astrociti del mesencefalo	Trattamento della retinite pigmentosa
Latvian	Cilvēka rekombinantais no vidussmadzeņu astrocītiem atvasinātais neirotrofiskais faktors	Retinitis pigmentosa ārstēšana
Lithuanian	Rekombinantinis iš žmogaus viduriniųjų smegenų astrocitų išskirtas neurotrofinis faktorius	Pigmentinio retinito gydymas
Maltese	Fattur newrotrofiku rikombinanti uman imnissel minn astroċiti mesenċefaliċi	Kura tar-retinite pigmentuża
Polish	Ludzki rekombinowany śródmózgowy czynnik neurotroficzny pochodzenia astrocytarnego	Leczenie retinopatii barwnikowej
Portuguese	Fator neurotrófico derivado de astrócitos mesencefálico recombinante humano	Tratamento da retinite pigmentosa
Romanian	Factor neutrofic uman recombinant derivat din astrocitele mezencefalice	Tratamentul retinitei pigmentare
Slovak	Ľudský rekombinantný mezencefalitický neurotrofický faktor derivovaný z astrocytov	Liečba retinitis pigmentosa

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humani rekombinantni mezencefalni, iz astrocitov pridobljen nevrotropni faktor	Zdravljenje pigmentozne retinopatije
Spanish	Factor neurotrófico derivado de astrocitos mesencefálicos recombinante humano	Tratamiento de retinosis pigmentaria
Swedish	Rekombinant human neurotropisk faktor från mesencefalon härrörande från astrocyter	Behandling av retinitis pigmentosa
Norwegian	Rekombinant human nevrotropisk faktor fra mesencephalon avledet fra astrocytter	Behandling av retinitis pigmentosa
Icelandic	Raðbrigða manna taugavaxtarþáttur úr miðheila afleiddur úr stjarnfrumum	Meðferð á retinitis pigmentosa