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Public summary of opinion on orphan designation

Recombinant truncated N-terminal fragment of human lens epithelium-derived growth factor for the treatment of retinitis pigmentosa

On 23 August 2017, orphan designation (EU/3/17/1908) was granted by the European Commission to Dorian Regulatory Affairs BV, the Netherlands, for recombinant truncated N-terminal fragment of human lens epithelium-derived growth 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 3.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 191,000 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 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 515,700,000 (Eurostat 2017).

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How is this medicine expected to work?

The way this medicine works is still being investigated, but laboratory studies have shown that injecting it into the eye can reduce damage to the retina. It is believed to reduce damage to retinal cells by encouraging the cells of the retina to produce protective proteins that help them resist damage from toxic substances or improperly formed proteins.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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 in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2017 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:

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 truncated N-terminal fragment of human lens epithelium-derived growth factor	Treatment of retinitis pigmentosa
Bulgarian	Рекомбинантен скъсен N-терминален фрагмент от растежен фактор, произхождащ от епителa на човешката леща	Лечение на пигментен ретинит
Croatian	Rekombinantni skraćeni N-terminalni fragment faktora rasta izvedenog iz epitela ljudske leće	Liječenje retinitisa pigmentoze
Czech	Rekombinantní zkrácený N- terminální fragment lidského růstového faktoru odvozeného od epitelu čočky	Léčba pigmentosní retinitidy
Danish	Rekombinant trunkeret N-terminal fragment af human linse epitel-deriveret vækstfaktor	Behandling af retinitis pigmentosa
Dutch	Recombinant getrunceerd N-terminaal fragment uit humaan lens epithelium-afgezonderde groei-factor	Behandeling van retinitis pigmentosa
Estonian	Rekombinantne inimese silmaläätse epiteeliumist lähtuva kasvufaktori N-terminaalosa lühendatud fragment	Pigmentoosse võrkkestapõletiku ravi
Finnish	Rekombinantti, typistetty, N-terminaalinen ihmisen linssin epiteeli kasvutekijän fragmentti	Verkkokalvorappeuman hoito
French	Fragment recombinant terminal-N codant du facteur de croissance derivé de cellules épithéliales du cristallin humain	Traitement de la rétinite pigmentaire
German	Rekombinantes gekürztes N-terminales Fragment des humanen Lens Epithelium-Derived Growth Faktors	Behandlung der Retinopathia Pigmentosa
Greek	Ανασυνδυασμένο περικομμένο Ν-τελικό τμήμα του ανθρώπινου αυξητικού παράγοντα που προέρχεται από το επιθηλιο του φακοtuberculosis	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Humán lencse epitéliumból származó növekedési faktor rekombináns csonkított N-terminális fragmense	Retinitis pigmentosa kezelése
Italian	Frammento recombinante troncato N-terminale del fattore di crescita derivato dall'epitelio del cristallino umano	Trattamento della retinite pigmentosa
Latvian	Rekombinants, saīsināts no cilvēka lēcas eptēlija iegūtā augšanas faktora N-termināla fragments	*Retinitis pigmentosa* ārstēšana
Lithuanian	Rekombinantinis sutrumpintas N-galinis fragmentas iš žmogaus lęšiuko epitelio išskirto augimo faktoriaus	Pigmentinio retinito gydymas
Maltese	Framment N-terminali maqtugħ rikombinanti tal-fattur uman tat-tkabbir lens derivat mill-epitelium	Kura tar-retinite pigmentuża

[1] At the time of designation

Language	Active ingredient	Indication
Polish	Rekombinowany skrócony N-terminalny fragment ludzkiego, pochodzącego z nabłonka soczewki czynnika wzrostu	Leczenie retinopatii barwnikowej
Portuguese	Fragmento N-terminal truncado recombinante do fator de crescimento derivado do epitélio do cristalino humano	Tratamento da retinite pigmentosa
Romanian	Fragment recombinant trunchiat N-terminal al factorului de creştere derivat din epiteliul cristalinului uman	Tratamentul retinitei pigmentare
Slovak	Rekombinantný skrátený N-terminálny fragment ľudského rastového faktora z epitélia očnej šošovky	Liečba retinitis pigmentosa
Slovenian	Rekombinantni skrajšan N-terminalni fragment rastnega faktorja iz epitelija humane leče	Zdravljenje pigmentozne retinopatije
Spanish	Fragmento N-terminal recombinante del factor de crecimiento derivado del epitelio de la lente humano truncado	Tratamiento de la retinosis pigmentaria
Swedish	Rekombinant trunkerat N-terminalt fragment av human lins-epithelium-framställd tillväxtfaktor	Behandling av retinitis pigmentosa
Norwegian	Rekombinant trunkert N-terminal fragment av human linse epitel-derivert vekst faktor	Behandling av retinitis pigmentosa
Icelandic	Raðbrigða stýfður N-terminal brot af manna linsu epithelium afleiddum vaxtarþætti	Meðferð á retinitis pigmentosa