



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

30 March 2015
EMA/COMP/43665/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Myriocin for the treatment of retinitis pigmentosa

On 12 February 2015, orphan designation (EU/3/15/1449) was granted by the European Commission to Nanovector s.r.l., Italy, for myriocin 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 2.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 123,000 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).



How is this medicine expected to work?

Myriocin works by blocking an enzyme called serine palmitoyl transferase, which is responsible for the production of a substance called ceramide. Ceramide is involved in inflammatory processes and programmed cell death (apoptosis).

Patients with retinitis pigmentosa are thought to have abnormally high levels of ceramide in the retina and therefore excessive death of retinal cells. When myriocin is given as eye drops, ceramide levels in the eye are expected to fall. This is expected to help retina cells to survive, thus delaying or preventing further loss of vision.

What is the stage of development of this medicine?

The effects of myriocin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with myriocin in patients with retinitis pigmentosa had been started.

At the time of submission, myriocin 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 January 2015 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:

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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	Myriocin	Treatment of retinitis pigmentosa
Bulgarian	Мυριοцин	Лечение на пигментен ретинит
Croatian	Miriocin	Liječenje retinitisa pigmentoze
Czech	Myriocin	Léčba pigmentosní retinitidy
Danish	Myriocin	Behandling af retinitis pigmentosa
Dutch	Myriocin	Behandeling van retinitis pigmentosa
Estonian	Müriotsiin	Pigmentoosse võrkkestapõletiku ravi
Finnish	Myriocin	Verkkokalvorappeuman hoito
French	Myriocine	Traitement de la rétinite pigmentaire
German	Myriocin	Behandlung der Retinopathia Pigmentosa
Greek	Μυριοσίνη	Θεραπεία της μελαγχρωστικής αμφιβληστροειδοπάθειας
Hungarian	Myriocin	Retinitis pigmentosa kezelése
Italian	Myriocin	Trattamento della retinite pigmentosa
Latvian	Miriocīns	Retinitis pigmentosa ārstēšana
Lithuanian	Myriocinas	Pigmentinio retinito gydymas
Maltese	Myriocin	Kura tar-retinite pigmentuża
Polish	Myriocin	Leczenie retinopatii barwnikowej
Portuguese	Miriocin	Tratamento da retinite pigmentosa
Romanian	Myriocin	Tratamentul retinitei pigmentare
Slovak	Myriocin	Liečba retinitis pigmentosa
Slovenian	Miriocin	Zdravljenje pigmentozne retinopatije
Spanish	Miriocin	Tratamiento de retinosis pigmentaria
Swedish	Myriocin	Behandling av retinitis pigmentosa
Norwegian	Myriocin	Behandling av retinitis pigmentosa
Icelandic	Mýriocín	Meðferð á retinitis pigmentosa

¹ At the time of designation