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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

N-({ Carbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluoro-phenyl)-ethylamino]-N-isobutyl-acetamide for the treatment of optic neuritis

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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 19 February 2014, orphan designation (EU/3/14/1248) was granted by the European Commission to Bionure Farma SL, Spain, for N-({ Carbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluoro-phenyl)-ethylamino]-N-isobutyl-acetamide for the treatment of optic neuritis.

What is optic neuritis?

Optic neuritis is a disease characterised by inflammation of the optic nerve, the nerve at the back of the eye that transmits signals to the brain. Optic neuritis is caused by an overactive immune system (the body's natural defences) which attacks the optic nerve leading to inflammation and death of the nerve cells. Symptoms often affect only one eye and include loss of vision, loss of colour vision and pain around the eye. Optic neuritis often develops in people with diseases of the immune system.

Optic neuritis is a long-term debilitating disease because of the visual loss and development of blindness.

What is the estimated number of patients?

At the time of designation, optic neuritis affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 people*, and is below the ceiling for orphan

*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 512,900,000 (Eurostat 2014).



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, there were no satisfactory methods specifically authorised in the EU for the treatment of optic neuritis. Only medicines to relieve the inflammation and symptoms were available, including corticosteroids (anti-inflammatory medicines) and medicines that reduce the activity of the immune system (the body's natural defences).

How is this medicine expected to work?

The way this medicine works is not clear, but it is expected to cross the blood-brain barrier, which separates the blood from the brain tissue. In the brain, this medicine is thought to protect nerve cells from damage and it has been shown to attach to certain proteins called SGK2, which help nerve cells to survive.

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 optic neuritis had been started.

At the time of submission, this medicine was not authorised anywhere in the EU for optic neuritis 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 2014 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	N-({ Carbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluorophenyl)-ethylamino]-N-isobutyl-acetamide	Treatment of optic neuritis
Bulgarian	N-({ карбамоилметил-[3-(2-оксо-пирролидин-1-ил)-пропил]-карбамоил}-метил)-2-[2-(2-флуоро-фенил)-етиламино]-N-изобутил-ацетамид	Лечение на оптичен неврит
Czech	N-({ karbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-karbamoyl}-methyl)-2-[2-(2-fluorofenyl)-ethylamino]-N-isobutyl-acetamid	Léčba optické neuritidy
Croatian	N-({ karbamoilmetil-[3-(2-okso-pirolidin-1-il)-propil]-karbamoil}-metil)-2-[2-(2-fluoro-fenil)-etilamino]-N-izobutil-acetamid	Liječenje optičkog neuritisa
Danish	N-({ Carbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluorofenyl)-ethylamino]-N-isobutyl-acetamid	Behandling af optisk neuritis
Dutch	N-({ carbamoylmethyl-[3-(2-oxo-pyrrolidine-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluorofenyl)-ethylamino]-N-isobutyl-acetamide	Behandeling van neuritis optica
Estonian	N-({ Karbamoüülmetyül-[3-(2-okso-pürrolidiin-1-üül)-propüül]-karbamoüül}-metüül)-2-[2-(2-fluoro-fenüül)-etüülamino]-N-isobutüül-atsetamiid	Nägemisnärvipiõletiku raviks
Finnish	N-({ karbamoyylimetyyli-[3-(2-okso-pyrrolidiini-1-yyli)-propyyli]-karbamoyyli}-metyyli)-2-[2-(2-fluorofenyli)-etyyliamino]-N-isobutyylimetyyli-asetamidi	Näköhermon tulehduksen hoito
French	N-({ carbamoylméthyl[3-(2-oxopyrrolidin-1-yl)propyl]carbamoyl}méthyl)-2-[2-(2-fluorophényl)éthylamino]-N-isobutylacétamide	Traitement de la névrite optique
German	N-({ Carbamoylmethyl-[3-(2-oxopyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluorophenyl)-ethylamino]-N-isobutylacetamid	Behandlung der optischen Neuritis
Greek	N-({ Καρβαμοϋλμεθυλ-[3-(2-οξο-πυρολιδιν-1-υλ)-προπυλ]-καρβαμοϋλ}-μεθυλ)-2-[2-(2-φθορο-φαινυλ)-εθυλαμινο]-N-ισοβουτυλ-ακεταμίδιο	Θεραπεία της οπτικής νευρίτιδας

¹ At the time of designation

Language	Active ingredient	Indication
Hungarian	N-({ karbamoilmetil-[3-(2-oxopirrolidin-1-il)-propil]-karbamoil}-metil)-2-[2-(2-fluorofenil)-etilamino]-N-izobutil-acetamid	Neuritis opticus kezelése
Italian	N-({ Carbamoilmetil-[3-(2-osso-pirrolidin-1-il)-propil]-carbamoil}-metil)-2-[2-(2-fluoro-fenil)-etilamino]-N-isobutil-acetamide	Trattamento della neurite ottica
Latvian	N-({ karbamoilmetil-[3-(2-okso-pirolidin-1-il)-propil]-karbamoil}-metil)-2-[2-(2-fluor-fenil)-etilamino]-N-izobutil-acetamīds	Redzes nerva neirīta ārstēšana
Lithuanian	N-({ karbamoilmetil-[3-(2-okso-pirolidin-1-il)-propil]-karbamoil}-metil)-2-[2-(2-fluoro-fenil)-etilamino]-N-izobutil-acetamidas	Optinio neurito gydymas
Maltese	N-({ Carbamoylmethyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-carbamoyl}-methyl)-2-[2-(2-fluoro-phenyl)-ethylamino]-N-isobutyl-acetamide	Kura ta' newrite ottika
Polish	N-({ karbamoilo-metylo-[3-(2-okso-pirolidyno-1-ylo)-propylo]-karbamoilo}-metylo)-2-[2-(2-fluoro-fenylo)-etylamino]-N-izobutylo-acetamid	Leczenie zapalenia nerwu wzrokowego
Portuguese	N-({ Carbamoilmetil-[3-(2-oxo-pirrolidina-1-il)-propil]-carbamoil}-metil)-2-[2-(2-fluoro-fenil)-etilamino]-N-isobutil-acetamida	Tratamento da nevrite óptica
Romanian	N-({ carbamoilmetil-[3-(2-oxo-pirolidin-1-il)-propil]-carbamoil}-metil)-2-[2-(2-fluoro-fenil)-etilamino]-N-izobutil-acetamidă	Tratamentul nevritei optice
Slovak	N-({ karbamoylmetyl-[3-(2-oxo-pyrrolidín-1-yl)-propyl]-karbamoyl}-metyl)-2-[2-(2-fluóro-fenyl)-etylamino]-N-izobutyl-acetamid	Liečba optickej neuritídy
Slovenian	N-({ karbamoilmetil-[3-(2-okso-pirolidin-1-il)-propil]-karbamoil}-metil)-2-[2-(2-fluoro-fenil)-etilamino]-N-izobutil-acetamid	Zdravljenje optičnega nevritisa
Spanish	N-({ Carbamoilmetil-[3-(2-oxo-pirrolidin-1-il)-propil]-carbamoil}-metil)-2-[2-(2-fluoro-fenil)-etilamino]-N-isobutil-acetamida	Tratamiento de la neuritis óptica
Swedish	N-({ karbamoylmetyl-[3-(2-oxo-pyrrolidin-1-yl)-propyl]-karbamoyl}-metyl)-2-[2-(2-fluoro-fenyl)-etylamino]-N-isobutyl-acetamid	Behandling av optisk neurit
Norwegian	N-({ karbamoylmetyl-[3-(2-okso-pyrrolidin-1-yl)-propyl]-karbamoyl}-metyl)-2-[2-(2-fluor-fenyl)-etylamino]-N-isobutyl-acetamid	Behandling av optikusnevritt
Icelandic	N-({ karbamóýlmetyl-[3-(2-oxó-pýrrólídín-1-ýl)-própyl]-karbamóýl}-metýl)-2-[2-(2-flúor-fenýl)-etylamínó]-N-ísóbútýl-acetamíð	Meðferð við sjóntaugarbólgu