28 November 2013
EMA/COMP/631492/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Synthetic 12 amino acid peptide designed after subcommissural organ-spondin for the treatment of spinal cord injury

On 13 November 2013, orphan designation (EU/3/13/1206) was granted by the European Commission to Neuronax SAS, France, for synthetic 12 amino acid peptide designed after subcommissural organ-spondin for the treatment of spinal cord injury.

What is spinal cord injury?

The spinal cord can be injured through accidents, such as damage to the back, or by internal causes such as tumours or bleeding within the spine putting pressure on the spinal cord. Injury to the spinal cord can damage the nerves that run through the cord and that branch out from it. This can stop the flow of nerve impulses between the brain and the body, resulting in loss of feeling, paralysis and even death, depending upon the severity of the injury and where it is located.

Spinal cord injury is a life-threatening disease that is debilitating in the long-term, because it can cause paralysis of the arms and legs and reduces life expectancy.

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

At the time of designation, spinal cord injury affected less than 4.2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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?

At the time of designation, methylprednisolone (a steroid) was authorised for the treatment of spinal cord injury in some countries in the EU. Methylprednisolone reduces the inflammation and pressure on the spinal cord that can occur after it is damaged. Patients with spinal cord injury could also have decompression surgery to reduce the pressure on the spine.

*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 512,200,000 (Eurostat 2013).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with spinal cord injury because it works in a different way to existing treatments and experimental studies show that it may encourage the survival and regrowth of nerve cells. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Subcommissural organ-spondin (SCO-spondin) is a substance secreted by the brain during its development before birth and is thought to play a role in the development of the nervous system. This medicine is a synthetic version of part of the SCO-spondin molecule. When introduced into the damaged spinal cord it is expected to encourage the survival and re-growth of nerve cells and their connections, thereby reducing the area of damage to the spinal cord and improving its recovery.

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 spinal cord injury had been started.

At the time of submission, synthetic 12 amino acid peptide designed after subcommissural organ-spondin was not authorised anywhere in the EU for spinal cord injury 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 October 2013 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:

Neuronax SAS
Biopôle Clermont Limagne
63360 Saint Beauzire
France
Tel. +33 473 449 430
E-mail: sgobron@neuronax.com

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	Synthetic 12 amino acids peptide designed after subcommissural organ spondin	Treatment of spinal cord injury
Bulgarian	Синтетичен дванадесет амино киселинен пептид структуриран като спондин от субкомисуралния орган	Лечение на гръбначно-мозъчни травми
Czech	Syntetický dvanáct aminokyselin obsahující peptid navržen dle subcommissurálního orgánového spondinu	Léčba míšního traumatu
Croatian	Sintetični peptid od 12 aminokiselina dizajniran prema spondinu subkomisuralnog organa	Liječenje ozljede kralježnične moždine
Danish	Syntetisk tolv aminosyrer peptid designet efter subcommissural orgel-spondin	Behandling af rygmarvslæsion
Dutch	Synthetische 12- aminozuren peptide ontworpen gebaseerd op subcommissuraal orgaan-spondine	Behandeling van ruggenmergletsel
Estonian	Sünteetiline 12-aminohappeline peptiid disainitud peale t *subcommissural organ spondin*	Seljaaju vigastuse ravi
Finnish	Synteettinen 12-aminohappoinen, subkommissuuraelimen spondiinin mukaan tehty peptidi	Selkäydinvamman hoito
French	Peptide synthétique de douze acides aminés conçu d'après la spondine sécrétée par l'organe sous commissural	Traitement des lesions de la moëlle épinière
German	Subcommissuralorgan-Spondin s nachempfundenes synthetisches Zwölf-Aminosäure-Peptid	Behandlung der Rückenmarkverletzung
Greek	Συνθετικό πεπτίδιο δώδεκα αμινοξέων σχεδιασμένο με βάση την σπονδίνη του υποσυνδεσμικού οργάνου του εγκεφάλου	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Tizenkét aminosavból álló, szubkomisszurális szerv spondinja alapján tervezett szintetikus peptid	Gerincvelő sérülés kezelése
Italian	Peptide sintetico di dodici aminoacidi progettato dopo subcommissural organo-spondina	Trattamento delle lesioni del midollo spinale
Latvian	Sintētisks divpadsmit aminoskābju peptīds veidots pēc subkommisurāla orgānu-spondīna	Mugurkaula traumu ārstēšana
Lithuanian	Sintetinis 12-os aminorūgščių baltymas skirtas po subkomisūrinio organo spondino	Nugaros smegenų sužalojimo gydymas

[1] At the time of designation

Language	Active ingredient	Indication
Maltese	Peptide sintetiku bi tnax-il aċidu amino mfassal fuq l-ispondin tal-organu subkommissurali	Kura ta' korriment tan-nerv qawwi li jgħaddi minn ġos-sinsla
Polish	Syntetyczny dwunastoaminokwasowy peptyd wzorowany na spondynie narządu podspoidłowego	Leczenie uszkodzenia rdzenia kręgowego
Portuguese	Peptídeo sintético de doze aminoácidos derivado da glicoproteína *subcommissural organ spondin*	Tratamento da lesão da medula espinal
Romanian	Peptidă de sinteză formată din 12 amino-acizi sintetizată după modelul spondinei secretată de organul subcomisural	Tratamentul leziunilor măduvei spinării
Slovak	Syntetický dvanásť aminokyselinový peptid navrhnutý podľa subkomisurálneho orgánového spondinu	Liečba poškodenia miechy
Slovenian	Sintetični dvanajst aminokislinski peptid zasnovan po spondinu subkomisurnega organa	Zdravljenje poškodbe hrbtenjače
Spanish	Péptido sintetico de doce amino ácidos desiñado segun el órgano subcomisural-espondina	Tratamiento de las lesiones de la médula espinal
Swedish	Syntetisk tolv-aminosyror-peptid utformad efter subcommissural organ-spondin	Behandling av ryggmärgsskada
Norwegian	Syntetisk tolv aminosyrer peptid utformet etter subcommissural organ-spondin	Behandling av ryggmargsskade
Icelandic	Tilbúinn tólf amínó sýru peptíð hannað eftir subcommissúral líffæra -spondín	Meðferð mænuskaða vegna slyss