13 October 2011
EMA/COMP/659363/2011
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

$NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ for the treatment of traumatic spinal cord injury

On 27 September 2011, orphan designation (EU/3/11/910) was granted by the European Commission to PHARMAXON, France, for $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ for the treatment of traumatic spinal cord injury.

## What is traumatic spinal cord injury?

Traumatic spinal cord injury is damage to the spinal cord caused by an accident, such as a blow to the back. Injury to the spinal cord can damage the nerves that run through the cord and branch out from it. This can stop the flow of nerve impulses between the brain and the rest of the body, resulting in the loss of sensation, paralysis and even death, depending on the severity and location of the injury.

The development of traumatic spinal cord injury can be divided into two phases: the acute phase and the recovery phase. During the acute phase (lasting for a few weeks after the injury) a process of inflammation starts, in which the damage spreads to the nerve cells surrounding the original site of injury, leading to the death of many of those nerve cells. In the recovery phase, the surviving nerve cells recover some of their function. The improvement usually continues for up to one year, after which the patient's condition tends not to improve any further.

Traumatic 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, traumatic spinal cord injury affected approximately 3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 152,000 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).

[*]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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
**Telephone** +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
**E-mail** info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

## 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 can also have decompression surgery to reduce the pressure on the spine.

The sponsor has provided sufficient information to show that $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ might be of significant benefit for patients with traumatic spinal cord injury because it works in a different way to existing treatments, and early studies show that it might improve the treatment of patients with this condition. These assumptions 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?

The medicine is made of a peptide (a chain of amino acids) intended to mimic the actions of polysialic acid (PSA). PSA is found on developing nerve cells, attached to a molecule called neural cell adhesion molecule (NCAM), and is thought to be involved in repairing damage to nerve cells. The medicine is expected to repair nerve damage through a number of mechanisms, such as increasing the amount of NCAM, inhibiting the action of certain cells and increasing the amount of certain fibres in scarred lesions.

## What is the stage of development of this medicine?

The effects of $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ 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 traumatic spinal cord injury had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for traumatic 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 8 July 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:

PHARMAXON
Grand Luminy Technopole case 922
Parc Scientifique de Luminy
13288 Marseille Cedex 09
France
Telephone: +33 4 86 94 85 10
Telefax: +33 4 91 82 84 85
E-mail: norreel@pharmaxon.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 | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Treatment of traumatic spinal cord injury |
| Bulgarian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Лечение на травматична увреда на гръбначния мозък |
| Czech | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Léčba míšního traumatu |
| Danish | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Behandling af traumatisk rygmarvslæsion |
| Dutch | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Behandeling van traumatisch ruggenmergletsel |
| Estonian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Traumaatilise seljaaju kahjustuse ravi |
| Finnish | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Traumaattisen selkäydinvamman hoito |
| French | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Traitement du traumatisme de la moëlle épinière |
| German | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Behandlung traumatischer Rueckenmarksverletzungen |
| Greek | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Θεραπεία τραύματος της σπονδυλικής στήλης |
| Hungarian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Traumás gerincvelő sérülés kezelése |
| Italian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Trattamento del trauma acuto della colonna vertebrale |
| Latvian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Muguras smadzeņu traumatiska bojājuma ārstēšana |
| Lithuanian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Nugaros smegenų trauminio pažeidimo gydymas |
| Maltese | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Kura ta' korriment trawmatiku tan-nerv qawwi li jgħaddi minn ġos-sinsla |
| Polish | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Leczenie pourazowego uszkodzenia rdzenia kręgowego |
| Portuguese | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Tratamento da lesão traumática da medula espinal |
| Romanian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Tratamentul leziunilor traumatice ale măduvei spinării |
| Slovak | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Liečba traumatického poškodenia miechy |
| Slovenian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Zdravljenje travmatske poškodbe hrbtenjače |
| Spanish | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Tratamiento de las lesiones espinales medulares traumáticas |

[1] At the time of designation

| Language | Active ingredient | Indication |
|---|---|---|
| Swedish | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Behandling av traumatisk ryggmärgsskada |
| Norwegian | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Behandling av traumatisk ryggmargsskade |
| Icelandic | $NH_2$-Cys-Ser-Ser-Val-Thr-Ala-Trp-Thr-Thr-Gly-Cys-Gly-$CONH_2$ | Meðferð mænuskaða vegna slyss |