



COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF 3-methoxy-pregnenolone for the treatment of spinal cord injury

On 4 December 2007, orphan designation (EU/3/07/511) was granted by the European Commission to MAPREG SAS, France, for 3-methoxy-pregnenolone for the treatment of spinal cord injury.

What is spinal cord injury?

The spinal cord is a cylindrical extension of the brain, contained within the bones of the spine. The spinal cord gives origin to the nerves that send information (sensations) to the brain, and distribute orders to the various parts of the body (mostly to the muscles). The spinal cord is protected by the bones of the spine, but it can be damaged by any external injury (trauma to the back) or by internal causes (a tumour or bleeding, for example). Injury to the spinal cord can damage the nerves, and stop the connection between the brain and the parts of the body that either receive orders from the brain or send information to it. Stopping this communication, results in lack of sensitivity, paralysis and even death, depending upon the intensity and level of the lesion and the structures damaged.

Spinal cord injury is life-threatening and chronically debilitating, due to increased mortality and severe complications (e.g. paralysis of the legs or of all limbs, respiratory difficulties, blood clots in the veins and the lungs, and repeated infections of the kidneys, the respiratory system and the urinary tract).

What are the methods of treatment available?

No satisfactory methods exist that were authorised at the time of application. Surgical intervention is often performed to decrease the pressure over the spine (decompression), but its role is controversial. High doses of methylprednisolone are also sometimes given.

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

Based on the information provided by the sponsor and previous knowledge of the Committee, spinal cord injury was considered to affect between 2.2 and 4.2 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 110,000-209,000 persons.

How is this medicinal product expected to act?

3-methoxy-pregnenolone is derived from a natural hormone (a substance that circulates in the blood and modifies bodily functions at a distance), called pregnenolone. 3-methoxy-pregnenolone is expected to help spinal cord cells, which are damaged after spinal cord injury occurs, to recover their structure and eventually their function.

* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

What is the stage of development of this medicinal product?

The effects of 3-methoxy-pregnelone were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with spinal cord injury were initiated.

The medicinal product was not authorised anywhere in the world for the treatment of spinal cord injury, or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 October 2006 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

Sponsor's contact details:

MAPREG SAS

CHU Bicêtre Bat Paul Langevin

78 rue du Général Leclerc

94275 Le Kremlin-Bicêtre Cedex

France

Telephone: +33 1 49 59 18 77

Telefax: +33 1 49 59 92 03

E-Mail: robela@kb.inserm.fr

Patients' associations contact points:

Associazione Luca Coscioni

via di Torre Argentina

76 - 00186 Roma

Italy

Telephone: +39 06 68 97 91

Telefax: +39 06 68 80 53 96

Danish Spinal Cord Injuries Association

Hans Knudsens Plads 1 A

DK 2100 København Ø

Denmark

Telephone: +45 39 29 35 55

Telefax: +45 39 29 39 48

E-mail: info@ryk.dk

I.W.A. – Irish Wheelchair Association

National headquarters

Áras Chúchulainn

Blackheath Drive

Clontarf

Dublin 3

Ireland

Telephone: +353 1 8186 400

Telefax: +33 1 8333 873

E-mail: info@iwa.ie

**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	3-methoxy-pregnenolone	Treatment of spinal cord injury
Bulgarian	3-метокси-прегненолон	Лечение на гръбначно-мозъчни травми
Czech	3- methoxy-pregnenolon	Léčba míšního traumatu
Danish	3-methoxy-pregnenolon	Behandling af rygmarsvlesion
Dutch	3-methoxy-pregnenolone	Behandeling van ruggenmergletsel
Estonian	3-metoksi-pregnenoloon	Seljaaju vigastuse ravi
Finnish	3-metoksi-pregnenoloni	Selkäydinvamman hoito
French	3-methoxy-pregnenolone	Traitement des lésions de la moëlle épinière
German	3-Methoxypregnenolon	Behandlung der Rückenmarkverletzung
Greek	3-μεθοξύ-πρεγνέλονη	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	3-metoxi-pregnenolon	Gerincvelő sérülés kezelése
Italian	3-metossi-pregnenolone	Tattamento delle lesioni del midollo spinale
Latvian	3-metoksi-pregnenolons	Mugurkaula traumā ārstēšana
Lithuanian	3-metoksi- pregnenolonas	Nugaros smegenų sužalojimo gydymas
Maltese	3-methoxy-pregnenolone	Kura ta' korriment tan-nerv qawwi li jghaddi minn ġos-sinla
Polish	3-metoksy-pregnenolon	Leczenie uszkodzenia rdzenia kręgowego
Portuguese	3-metoxi-pregnenolona	Tratamento da lesão da medula espinal
Romanian	3-metoxi pregnenolonă	Tratamentul leziunilor măduvei spinării
Slovak	3-metoxi-pregnenólon	Liečba poškodenia miechy
Slovenian	3-metoksi-pregnenolon	Zdravljenje poškodbe hrbtenjače
Spanish	3-metoxi-pregnenolona	Tratamiento de las lesiones de la médula espinal
Swedish	3-methoxy-pregnenolon	Behandling av ryggmärgsskada
Norwegian	3-metoksy-pregnenolon	Behandling av ryggmargsskade
Icelandic	3-methoxy-pregnenólón	Meðferð mænuskaða vegna slyss