



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of guanabenz for the treatment of traumatic spinal cord injury

On 29 April 2009, orphan designation (EU/3/09/625) was granted by the European Commission to Acure Pharma AB, Sweden, for guanabenz 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 and kill the nerve cells 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 the loss of feeling, paralysis and even death, depending upon the severity of the injury and where it is located.

The development of traumatic spinal cord injury is divided in 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. In the recovery phase, the surviving nerve cells recover part of their function. The improvement usually continues for up to a year, after which the patient's condition does not improve any further.

Traumatic spinal cord injury is a life-threatening and long-term debilitating disease because it can cause paralysis of the arms and legs and reduce life expectancy.

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

At the time of designation, traumatic spinal cord injury affected approximately 0.6 people in 10,000 per year in the European Union (EU)*. This is equivalent to a total of around 30,000 people per year, which was considered to be below the threshold for orphan designation. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for orphan drug 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 happen after it is damaged. Patients with spinal cord injury can also have surgery to reduce the pressure on the spine. The sponsor has provided sufficient information to show that guanabenz might be of significant benefit for the patients because it works in a different way to existing treatment. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

*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 504,800,000 (Eurostat 2009).

How is this medicine expected to work?

Guanabenz has been available as a medicine to treat high blood pressure since the 1980s. In traumatic spinal cord injury, it is expected to work during the acute phase of the injury by reducing inflammation. It is thought to work by blocking enzymes called cyclo-oxygenase, lipoxygenase and nitric oxide synthase, which produce substances that are involved in the inflammation process. Guanabenz is not expected to work in the recovery phase of the disease because it has no effect on the regeneration of nerve cells.

What is the stage of development of this medicine?

The effects of guanabenz have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with traumatic spinal cord injury had been started.

At the time of submission, guanabenz was not authorised anywhere in the EU for traumatic spinal cord injury or designated as 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 4 March 2009 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 Community) 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.

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

Language	Active ingredient	Indication
English	Guanabenz	Treatment of traumatic spinal cord injury
Bulgarian	Guanabenz	Лечение на травматична увреда на гръбначния мозък
Czech	Guanabenz	Léčba míšního traumatu
Danish	Guanabenz	Behandling af traumatisk rygmarslæsion
Dutch	Guanabenz	Behandeling van traumatisch ruggenmergletsel
Estonian	Guanabenz	Traumaatilise seljaaju kahjustuse ravi
Finnish	Guanabentsi	Traumaattisen selkäydinvamman hoito
French	Guanabenz	Traitement du traumatisme de la moëlle épinière
German	Guanabenz	Behandlung traumatischer Rueckenmarksverletzungen
Greek	Guanabenz	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Guanabenz	Traumás gerincvelő sérülés kezelése
Italian	Guanabenz	Trattamento del trauma acuto della colonna vertebrale
Latvian	Guanabenzs	Muguras smadzeņu traumatiska ievainojuma ārstēšana
Lithuanian	Guanabenzas	Nugaros smegenų trauminio pažeidimo gydymas
Maltese	Guanabenz	Kura ta' korrimient trawmatiku tan-nerv qawwi li jghaddi minn gos-sinsla
Polish	Guanabenz	Leczenie pourazowego uszkodzenia rdzenia kręgowego
Portuguese	Guanabenz	Tratamento da lesão traumática da medula espinal
Romanian	Guanabenz	Tratamentul leziunilor traumatice ale măduvei spinării
Slovak	Guanabenz	Liečba traumatického poškodenia miechy
Slovenian	Guanabenz	Zdravljenje travmatske poškodbe hrbtenjače
Spanish	Guanabenz	Tratamiento de las lesiones espinales medulares traumáticas
Swedish	Guanabenz	Behandling av traumatisk ryggmärgsskada