



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3 October 2013
EMA/COMP/178558/2004 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Deferoxamine mesylate for the treatment of traumatic spinal cord injury

First publication	16 January 2008
Rev.1: sponsor's name change	19 August 2009
Rev.2: withdrawal from the Community Register	1 July 2013
Rev.3: administrative update	3 October 2013
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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2013 on request of the Sponsor.

On 20 October 2004, orphan designation (EU/3/04/231) was granted by the European Commission to Neuraxo Biopharmaceuticals GmbH, Germany, for deferoxamine mesilate for the treatment of traumatic spinal cord injury.

Neuraxo Biopharmaceuticals GmbH changed name to SCT Spinal Cord Therapeutics GmbH in May 2009.

What is spinal cord injury?

The spinal cord is a cylindrical extension of the brain that passes through the vertebral canal that is formed by the bones of the spine. The spinal cord is formed by nerve fibres that send information (sensations) to the brain and distribute orders from it to the various parts of the body (mostly to the muscles). The spinal cord is protected by the spine bones, but it can be damaged by any injury acting directly on it or indirectly through surrounding structures (bones, vessels, etc.). An injury to the spinal cord could damage the nerves and therefore stop the connection between the brain and the distal structures of the body, resulting in lack of sensation, paralysis and even death, depending on the extent and level of the lesion and the structures damaged.



Spinal cord injury is life-threatening and chronically debilitating, due to the severe complications (e.g. paralysis of all four limbs, respiratory difficulties, blood clots, infections etc.).

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

At the time of designation, spinal cord injury affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 28,000 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?

No satisfactory methods for the treatment of spinal cord injury existed at the time of application.

How is this medicine expected to work?

When the spinal cord is injured, the normal process of tissue repair results in the formation of a scar. However, the scar will not allow nerve growth and the formation of new connections that would restore the normal flux of information in the spinal cord. Deferoxamine binds to iron, one of the elements required to produce collagen, the major component of scar tissue, making it less available to the tissues. Therefore, deferoxamine would stop scar formation by decreasing the availability of iron, which is necessary for the production of the scar. The inhibition of the production of the scar would provide an opportunity to the damaged nerves to restore first the connections and then the functions.

What is the stage of development of this medicine?

The effects of deferoxamine mesilate were evaluated in experimental models.

Deferoxamine mesilate was not marketed anywhere worldwide for spinal cord injury or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 September 2004 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

*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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 464,200,000 (Eurostat 2004).

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:

SCT Spinal Cord Therapeutics GmbH
Max-Planck-Straße 15a
D-40699 Erkrath
Germany
Tel.: +49 211 61 78 510
Fax: +49 211 61 78 5150
E-mail: info@sctpx.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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Deferoxamine mesilate	Treatment of traumatic spinal cord injury
Czech	Deferoxamin mesilas	Léčba míšního traumatu
Danish	Deferoxaminmesilat	Behandling af traumatisk rygmarslæsion
Dutch	Deferoxaminemesilaat	Behandeling van traumatisch ruggenmergletsel
Estonian	Deferoksamiinmesülaat	Traumaatilise seljaaju kahjustuse ravi
Finnish	Deferoksamiinimesylaatti	Selkäydinvamman hoito
French	Mésilate de déferoxamine	Traitement du traumatisme de la moëlle épinière
German	Deferoxaminmesylat	Behandlung von akutem Rückenmarktrauma
Greek	Μεσυλική δεφεροξαμίνη	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Deferaxamin mesylat	Traumás gerincvelő sérülés kezelése
Italian	Deferossamina mesilato	Trattamento del trauma acuto della colonna vertebrale
Latvian	Deferoksamīna mesilāts	Muguras smadzeņu traumatiska ievainojuma ārstēšana
Lithuanian	Deferoksamino mezilatas	Nugaros smegenų trauminio pažeidimo gydymas
Maltese	Deferoxamine mesylate	Treatment of traumatic spinal cord injury
Polish	Deferoksaminy metanosulfonian	Leczenie pourazowego uszkodzenia rdzenia kręgowego
Portuguese	Mesilato de deferroxamina	Tratamento da lesão traumática da medula espinal
Slovak	Deferoxamini mesilas	Liečba traumatického poškodenia miechy
Slovenian	Deferoksamin mezilat	Zdravljenje travmatske poškodbe hrbtenjače
Spanish	Mesilato de deferroxamina	Tratamiento de las lesiones espinales medulares traumáticas
Swedish	Deferoxaminmesylat	Behandling av traumatisk ryggmärgsskada
Norwegian	Deferoksaminmesilat	Behandling av traumatisk ryggmargsskade
Icelandic	Deferoxamín mesýlat	Meðferð mænuskaða vegna slyss

¹ At the time of designation