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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Filgrastim for the treatment of spinal cord injury

First publication	23 April 2009
Rev.1: transfer of sponsorship	27 June 2014
Rev.2: transfer of sponsorship	12 March 2015
<b>Disclaimer</b> 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.	

On 7 November 2008, orphan designation (EU/3/08/580) was granted by the European Commission to Sygnis Bioscience GmbH & Co. KG, Germany, for filgrastim for the treatment of spinal cord injury.

The sponsorship was transferred to Dr Ulrich Bogdahn, Germany, in April 2014 and subsequently to NeuroVision Pharma GmbH, Germany, in February 2015.

### 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 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.

Spinal cord injury is life-threatening and chronically debilitating 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, spinal cord injury affected between 2.2 and 4.2 in 10,000 people in the European Union (EU). This was equivalent to a total of between 111,000 and 211,000 people<sup>\*</sup>, and is

<sup>\*</sup>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. At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).



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 submission of the application for orphan drug designation, methylprednisolone (a steroid) was authorised for the treatment of spinal cord in some countries in the Community. Methylprednisolone reduces inflammation and pressure on the spinal cord. At the time of submission of the application for orphan drug designation surgical intervention was often performed to decrease the pressure over the spine (decompression), but its role was controversial.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that filgrastim might be of potential significant benefit for the treatment of spinal cord injury because it has a new mechanism of action. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

### **How is this medicine expected to work?**

Filgrastim is a copy of a human protein called granulocyte colony stimulating factor (G-CSF), which may reduce cell death and promote cell division. In the treatment of spinal cord injury, filgrastim is expected to protect nerve cells in the spinal cord from death, thus limiting the effects of the injury.

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

The active ingredient is already authorised in the EU for treatment of other conditions in the field of haematology.

The effects of filgrastim have been evaluated in experimental models.

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

Filgrastim was not authorised anywhere worldwide for the treatment of spinal cord 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 10 September 2008 recommending the granting of this designation.

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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:

NeuroVision Pharma GmbH  
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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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Filgrastim	Treatment of spinal cord injury
Bulgarian	Филграстим	Лечение на гръбначно-мозъчни травми
Croatian	Filgrastim	Liječenje ozljede kralježnične moždine
Czech	Filgrastim	Léčba míšního traumatu
Danish	Filgrastim	Behandling af rygmarsvslæsion
Dutch	Filgrastim	Behandeling van ruggenmergletsel
Estonian	Filgastiim	Seljaaju vigastuse ravi
Finnish	Filgrastiimi	Selkäydinvamman hoito
French	Filgrastim	Traitement des lésions de la moëlle épinière
German	Filgrastim	Behandlung der Rückenmarkverletzung
Greek	Φιλγραστιμη	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Filgrasztim	Gerincvelő sérülés kezelése
Italian	Filgrastim	Trattamento delle lesioni del midollo spinale
Latvian	Filgrastims	Mugurkaula traumā ārstēšana
Lithuanian	Filgrastimas	Nugaros smegenų sužalojimo gydymas
Maltese	Filgrastim	Kura ta' korriment tan-nerv qawwi li jgħaddi minn ǧos-sinsla
Polish	Filgrastym	Leczenie uszkodzenia rdzenia kręgowego
Portuguese	Filgrastim	Tratamento da lesão da medula espinal
Romanian	Filgrastim	Tratamentul leziunilor măduvei spinării
Slovak	Filgrastim	Liečba poškodenia miechy
Slovenian	Filgrastim	Zdravljenje poškodbe hrbtenjače
Spanish	Filgrastim	Tratamiento de las lesiones de la médula espinal
Swedish	Filgrastim	Behandling av ryggmärgsskada
Norwegian	Filgrastim	Behandling av ryggmargsskade
Icelandic	Filgrastím	Meðferð mænuskaða vegna slyss

<sup>1</sup> At the time of transfer of sponsorship