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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Filgrastim for the treatment of amyotrophic lateral sclerosis

First publication	10 July 2008
Rev.1: transfer of sponsorship	27 June 2014
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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.

On 1 April 2008, orphan designation (EU/3/08/532) was granted by the European Commission to Sygnis Bioscience GmbH & Co. KG, Germany, for filgrastim for the treatment of amyotrophic lateral sclerosis.

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

What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis is a progressive disease of the nervous system. Amyotrophic lateral sclerosis occurs when specific nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate. The loss of these so-called motor neurons causes the muscles under their control to weaken and waste away, leading to paralysis. Amyotrophic lateral sclerosis varies from patient to patient, depending on which muscles weaken first. Symptoms may include tripping and falling, loss of motor control in hands and arms, difficulty in speaking, swallowing and/or breathing, persistent fatigue, and twitching and cramping. Amyotrophic lateral sclerosis strikes in mid-life. Men are about one-and-a-half times more likely to have the disease as women. Amyotrophic lateral sclerosis is chronically debilitating and life-threatening.

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

At the time of designation, amyotrophic lateral sclerosis affected approximately 0.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 35,000 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?

A medicinal product called riluzole was authorised for the condition in the Community at the time of submission of the application for orphan drug designation. Filgrastim might be of potential significant benefit for the treatment of amyotrophic lateral sclerosis. The benefit 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?

Granulocyte-colony stimulating factor acts as a growth factor in the body. Growth factors stimulate cells to proliferate or to gain specific functions. Filgrastim stimulates building and function of white blood cells called granulocytes. Granulocytes are important for the immune system as they are the cells responsible for destroying bacteria and external agents that enter the body.

The mechanism of action of filgrastim in amyotrophic lateral sclerosis is not fully understood, but it is thought that it would protect nerve cells and prevent nerve cells from deteriorating as seen in amyotrophic lateral sclerosis patients.

What is the stage of development of this medicine?

The evaluation of the effects of filgrastim in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with amyotrophic lateral sclerosis were initiated.

Filgrastim was not authorised anywhere worldwide for treatment of amyotrophic lateral sclerosis 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 6 February 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.

*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).

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:

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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[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Filgrastim	Treatment of amyotrophic lateral sclerosis
Bulgarian	Филграстим	Лечение на амиотрофична латерална склероза
Croatian	Filgrastim	Liječenje amiotrofične lateralne skleroze
Czech	Filgrastim	Léčba amyotrofické laterální sklerózy
Danish	Filgrastim	Behandling af amyotrofisk lateralsklerose
Dutch	Filgrastim	Behandeling van amyotrofe lateraalsclerose
Estonian	Filgastriim	Amüotroofilise lateraalskleroosi ravi
Finnish	Filgrastiimi	Amylotrofisen lateraaliskleroosin hoito
French	Filgrastim	Traitement de la sclérose latérale amyotrophique
German	Filgrastim	Zur Behandlung der amyotrophischen Lateralsklerose
Greek	Φιλγραστίμη	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Filgrasztim	Amyotrophiás lateral sclerosis kezelése
Italian	Filgrastim	Trattamento della sclerosi laterale amiotrofica
Latvian	Filgrastims	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Filgrastimas	Šoninės amiotrofinės sklerozės gydymas
Maltese	Filgrastim	Kura tas-sklerosi laterali amjotrofika
Polish	Filgrastym	Leczenie stwardnienia bocznego zanikowego
Portuguese	Filgrastim	Tratamento da esclerose lateral amiotrófica
Romanian	Filgrastim	Tratamentul sclerozei laterale amiotrofice
Slovak	Filgrastim	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Filgrastim	Zdravljenje amiotrofične lateralne skleroze
Spanish	Filgrastim	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Filgrastim	Behandling av amyotrofisk lateralskleros
Norwegian	Filgrastim	Behandling av amyotrofisk lateralsklerose
Icelandic	Fílgrastím	Meðferð við blandaðri hreyfitaugahrörnun

[1] At the time of transfer of sponsorship