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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human vascular endothelial growth factor for the treatment of amyotrophic lateral sclerosis

First publication	24 February 2010
Rev.1: sponsor's name change	29 April 2014

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 29 January 2010, orphan designation (EU/3/09/711) was granted by the European Commission to NeuroNova AB, Sweden, for recombinant human vascular endothelial growth factor for the treatment of amyotrophic lateral sclerosis.

In March 2014, NeuroNova AB changed name to Newron Sweden AB.

What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis (ALS) is a progressive disease of the nervous system, where the nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate. This causes the muscles under their control to weaken and waste away, leading to paralysis. The symptoms of ALS vary from patient to patient, depending on which muscles weaken first, and include tripping up, falling over, loss of control of hand and arm movement, difficulty speaking, swallowing and breathing, persistent tiredness, twitching and cramping. ALS usually starts in mid-life. Men are about one-and-a-half times more likely to develop the disease than women.

ALS is a debilitating disease, due to muscle deterioration, and a life-threatening disease because it affects the muscles that are used to breathe.

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

At the time of designation, ALS affected between 0.8 and 1 in 10,000 people in the European Union (EU). This was equivalent to a total of between 41,000 and 51,000 people*, and is below the threshold for orphan designation, which is 5 people in 10,000. 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 designation, there was one medicine called riluzole authorised for ALS in the EU.
The sponsor has provided sufficient information to show that recombinant human vascular endothelial growth factor might be of significant benefit for patients with ALS because it works in a different way to existing treatment and early studies in experimental models indicate that it might improve the treatment of patients with the disease. These assumptions will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Recombinant human vascular endothelial growth factor is a copy of a natural substance called vascular endothelial growth factor (VEGF). In the body, VEGF stimulates the development of blood vessels. The product is expected to be injected directly into the nervous system (in the ventricles of the brain), where it is expected to stimulate the growth of blood vessels to ensure better blood supply to the nerve cells, thus helping with their survival. The product is also expected to support the growth of new nerve cells.

What is the stage of development of this medicine?

The effects of recombinant human vascular endothelial growth factor have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the designated product in patients with ALS had been started.

At the time of submission, recombinant human vascular endothelial growth factor was not authorised anywhere in the EU for ALS or designated as an 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 5 November 2009 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 Community) 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 506,300,000 (Eurostat 2010).

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, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant human vascular endothelial growth factor	Treatment of amyotrophic lateral sclerosis
Bulgarian	Рекомбинантен човешки съдов ендотелен растежен фактор	Лечение на амиотрофична латерална склероза
Czech	Rekombinantní lidský vaskulární endoteliální růstový faktor	Léčba amyotrofické laterální sklerózy
Danish	Rekombinant human vaskulær endothelial vækstfaktor	Behandling af amyotrofisk lateralsklerose
Dutch	Recombinant humaan vasculair endotheliale groeifactor	Behandeling van amyotrofe lateraalsclerose
Estonian	Rekombinantne inimese vaskulaarse endoteeli kasvufaktori	Amüotroofilise lateraalskleroosi ravi
Finnish	Rekombinanttitekniikalla tuotettu ihmisen verisuonten endoteliaalinen kasvutekijä	Amyotrofisen lateraaliskleroosin hoito
French	Facteur de croissance de l'endothélium vasculaire humain recombinant (VEGF)	Traitement de la sclérose latérale amyotrophique
German	Rekombinanter humaner Vaskulärer Endothelialer Wachstumsfaktor	Behandlung der amyotrophen Lateralsklerose
Greek	Ανασυνδυασμένος ανθρώπινος Αγγειακός ενδοθηλιακός αυξητικός παράγοντας	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Rekombináns humán vascularis endothelialis növekedési faktor	Amyotrophiás lateral sclerosis kezelése
Italian	Fattore di crescita endoteliale ricombinante umano	Trattamento della sclerosi laterale amiotrofica
Latvian	Rekombinēts cilvēka asinsvadu endotēlija augšanas faktors	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Žmogaus rekombinantinis kraujagyslių endotelio augimo faktorius	Šoninės amiotrofinės sklerozės gydymas
Maltese	Fattur tat-tkabbir vaskulari endoteljali rikombinanti uman	Kura tas-sklerosi laterali amjotrofika
Polish	Rekombinowany ludzki czynnik wzrostu śródłbonka naczyń	Leczenie stwardnienia bocznego zanikowego
Portuguese	Factor de crescimento endothelial vascular humano recombinante	Tratamento da esclerose lateral amiotrófica
Romanian	Factor de creştere endotelial vascular uman recombinant	Tratamentul sclerozei laterale amiotrofice
Slovak	Rekombinantný ľudský cievny endoteliálny rastový faktor	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Rekombinantni humani rastni faktor žilnega endotelija	Zdravljenje amiotrofične lateralne skleroze
Spanish	Factor de crecimiento endotelial vascular recombinante humano	Tratamiento de la esclerosis lateral amiotrófica

Language	Active ingredient	Indication
Swedish	Rekombinant human vaskulär endotelial tillväxtfaktor	Behandling av amyotrofisk lateralskleros
Norwegian	Rekombinant human vaskulær endotelial vekstfaktor	Behandling av amyotrofisk lateralsklerose
Icelandic	Raðbrigða manna æðaþels vaxtarþáttur	Meðferð við blandaðri hreyfitaugahrörnun