27 May 2016
EMA/COMP/235531/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human cerebral dopamine neurotrophic factor for the treatment of amyotrophic lateral sclerosis

On 28 April 2016, orphan designation (EU/3/16/1652) was granted by the European Commission to Herantis Pharma Plc, Finland, for recombinant human cerebral dopamine neurotrophic factor for the treatment of amyotrophic lateral sclerosis.

What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis (ALS) is a progressive disease of the nervous system, where certain nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate, causing loss of muscle function and paralysis. The exact causes are unknown but are believed to include genetic and environmental factors. The symptoms of ALS vary depending on which muscles weaken first, and include loss of balance, loss of control of hand and arm movement, and difficulty speaking, swallowing and breathing. ALS usually starts in mid-life and men are more likely to develop the disease than women.

ALS is a long-term debilitating and life-threatening disease because of the gradual loss of function and its paralysing effect on muscles used for breathing, which usually leads to death due to respiratory failure.

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

At the time of designation, ALS affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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).

[*]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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What treatments are available?

At the time of designation, riluzole was authorised in the EU for the treatment of patients with ALS. Patients also received supportive treatment to temporarily relieve the symptoms of the disease, such as physiotherapy and speech therapy.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with the condition because early studies in experimental models suggest that it may improve muscle function, when compared with similar early studies of the currently authorised medicine. This assumption 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?

This medicine is made of a protein called cerebral dopamine neurotrophic factor (CDNF). CDNF is thought to play a role in the working of the endoplasmic reticulum, a structure inside cells involved among others in the correct folding of proteins. If this mechanism does not work properly, cells become 'stressed' and die. In patients with ALS, this is thought to contribute to the death of the nerve cells that control movement, and thus to the various signs and symptoms of the disease. CDNF is expected to have a protective function in nerve cells, reducing the stress and thus improving the symptoms of the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of this medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with ALS had been started.

At the time of submission, this medicine 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 23 March 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Recombinant human cerebral dopamine neurotrophic factor	Treatment of amyotrophic lateral sclerosis
Bulgarian	Човешки рекомбинантен мозъчен допамин невротрофичен фактор	Лечение на амиотрофична латерална склероза
Croatian	Rekombinantni ljudski moždani dopamin neurotrofni faktor	Liječenje amiotrofične lateralne skleroze
Czech	Lidský rekombinantní mozkový dopaminový neurotrofický faktor	Léčba amyotrofické laterální sklerózy (ALS)
Danish	Human rekombinant cerebral dopamin neurotrofisk faktor	Behandling af amyotrofisk lateralsklerose
Dutch	Humaan recombinant cerebrale dopamine neurotrofe factor	Behandeling van amyotrofe lateraalsclerose
Estonian	Rekombinantne inimese aju dopamiini neurotroofne faktor	Amüotroofilise lateraalskleroosi ravi
Finnish	Ihmisaivojen rekombinantti dopamiinin neurotrofinen tekijä	Amyotrofisen lateraaliskleroosin hoito
French	Facteur humain recombinant neurotrophique de la dopamine cérébrale	Traitement de la sclérose latérale amyotrophique
German	Rekombinanter humaner zerebraler Dopamin neurotropher Faktor	Behandlung der amyotrophen Lateralsklerose
Greek	Ανασυνδυασμένος ανθρώπινος εγκεφαλικός νευροτροφικός παράγοντας ντοπαμίνης	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Rekombináns humán agyi dopamin neurotrofikus faktor	Amyotrophiás lateral sclerosis kezelése
Italian	fattore neurotrofico cerebrale recombinante umano attivo sui neuroni dopaminergici	Trattamento della sclerosi laterale amiotrofica
Latvian	Rekombinants cilvēka cerebrālais dopamīna neirotrofiskais faktors	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Rekombinantinis žmogaus smegenų dopamino neurotrofinis faktorius	Šoninės amiotrofinės sklerozės gydymas
Maltese	Fattur rikombinanti uman newrotrofiku tad-dopamina tal-moħħ	Kura tas-sklerosi laterali amjotrofika
Polish	ludzki rekombinowany mózgowy dopaminowy czynnik neurotroficzny	Leczenie stwardnienia bocznego zanikowego
Portuguese	Fator neurotrófico da dopamina cerebral recombinante humano	Tratamento da esclerose lateral amiotrófica
Romanian	Factor uman recombinant neurotrofic al dopaminei cerebrale	Tratamentul sclerozei laterale amiotrofice
Slovak	Ľudský rekombinantný mozgový dopamínový neurotrofický faktor	Liečba amyotrofickej laterálnej sklerózy

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Humani rekombinantni nevrotropni faktor cerebralnega dopamine	Zdravljenje amiotrofične lateralne skleroze
Spanish	Factor neurotrófico de la dopamina-cerebral humano recombinante	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Rekombinant human cerebral dopamin neurotropisk faktor	Behandling av amyotrofisk lateralskleros
Norwegian	Rekombinant human cerebral dopamin nevrotropisk faktor	Behandling av amyotrofisk lateralsklerose
Icelandic	Raðbrigða manna heila dópamín taugavaxtarþáttur	Meðferð við blandaðri hreyfitaugahrörnun