EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Edaravone for the treatment of amyotrophic lateral sclerosis

On 16 December 2014, orphan designation (EU/3/14/1399) was granted by the European Commission to Treeway B.V., the Netherlands, for edaravone 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 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?

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).

What treatments are available?

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

*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 511,100,000 (Eurostat 2014).

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The sponsor has provided sufficient information to show that edaravone might be of significant benefit for patients with the condition based on data from early studies that showed potential favourable effects of using edaravone in combination with riluzole treatment. 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?

Damage to nerve cells in ALS appears to have several causes but there is evidence that it may involve damage to nerves caused by toxic molecules containing oxygen. In some patients this is associated with a defect in the gene responsible for producing the enzyme called superoxide dismutase (SOD), which causes the enzyme to clump together inside nerve cells. This leads to inflammation and kills the affected nerve cells. Edaravone is expected to act as an antioxidant, a molecule that can prevent damage to nerve cells caused by oxygen-containing molecules, and also blocks the clumping together of SOD in the nerves and so reduces inflammation.

Edaravone has been authorised in Japan to reduce nerve damage caused by acute ischaemic stroke (stroke caused by failure of the blood supply to part of the brain).

What is the stage of development of this medicine?

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

At the time of submission, no clinical trials with edaravone in patients with ALS were ongoing.

At the time of submission, edaravone was not authorised anywhere in the EU for ALS. Orphan designation had been granted in Japan for ALS.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 November 2014 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:

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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	Edaravone	Treatment of amyotrophic lateral sclerosis
Bulgarian	Едаравон	Лечение на амиотрофична латерална склероза
Croatian	Edaravon	Liječenje amiotrofične lateralne skleroze
Czech	Edaravon	Léčba amyotrofické laterální sklerózy
Danish	Edaravon	Behandling af amyotrofisk lateralsklerose
Dutch	Edaravon	Behandeling van amyotrofe lateraalsclerose
Estonian	Edaravoon	Amüotroofilise lateraalskleroosi ravi
Finnish	Edaravoni	Amyotrofisen lateraaliskleroosin hoito
French	Edaravone	Traitement de la sclérose latérale amyotrophique
German	Edaravon	Behandlung der amyotrophen Lateralsklerose
Greek	Ενταραβόνη	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Edaravon	Amyotrophiás lateral sclerosis kezelése
Italian	Edaravone	Trattamento della sclerosi laterale amiotrofica
Latvian	Edaravons	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Edaravonas	Šoninės amiotrofinės sklerozės gydymas
Maltese	Edaravone	Kura tas-sklerosi laterali amjotrofika
Polish	Edarawon	Leczenie stwardnienia bocznego zanikowego
Portuguese	Edaravona	Tratamento da esclerose lateral amiotrófica
Romanian	Edaravonă	Tratamentul sclerozei laterale amiotrofice
Slovak	Edaravón	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Edaravon	Zdravljenje amiotrofične lateralne skleroze
Spanish	Edaravona	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Edaravon	Behandling av amyotrofisk lateralskleros
Norwegian	Edaravon	Behandling av amyotrofisk lateralsklerose
Icelandic	Edaravón	Meðferð við blandaðri hreyfitaugahrörnun

[1] At the time of designation