



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

talampanel for the treatment of amyotrophic lateral sclerosis

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in August 2010 on request of the sponsor.

On 12 June 2009, orphan designation (EU/3/09/646) was granted by the European Commission to Teva Pharma GmbH, Germany, for talampanel 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 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 and life-threatening disease because it affects the muscles that are used to breathe.

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 is equivalent to a total of around 50,000 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.

* 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. This represents a population of 504,800,000 (Eurostat 2009).



The sponsor has provided sufficient information to show that talampanel might be of significant benefit for patients with ALS because it works in a different way to existing treatment and might represent an alternative way to treat ALS. 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?

Although the cause of ALS is largely unknown, high amounts of the neurotransmitter glutamate have been seen in patients with the disease. Neurotransmitters are naturally-occurring chemicals in the nervous system that allow nerve cells to communicate with each other. Very high levels of glutamate can lead to the damage and death of nerve cells in the brain and spinal cord. Talampanel is expected to work by blocking a type of receptor in the brain called AMPA receptors, to which glutamate normally attaches. By attaching to these receptors, talampanel is expected to block the action of glutamate, reducing the damage to nerve cells.

What is the stage of development of this medicine?

The effects of talampanel have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with ALS were ongoing.

At the time of submission, talampanel was not authorised anywhere in the EU for ALS. Orphan designation of talampanel had been granted in the United States of America for ALS.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 May 2009 recommending the granting of this designation.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Talampanel	Treatment of amyotrophic lateral sclerosis
Bulgarian	Талампанел	Лечение на амиотрофична латерална склероза
Czech	Talampanel	Léčba amyotrofické laterální sklerózy
Danish	Talampanel	Behandling af amyotrofisk lateralsklerose
Dutch	Talampanel	Behandeling van amyotrofe lateraalsclerose
Estonian	Talampaneel	Amüotroofilise lateraalskleroosi ravi
Finnish	Talampaneeli	Amyotrofisen lateraalskleroosin hoito
French	Talampanel	Traitement de la sclérose latérale amyotrophique
German	Talampanel	Behandlung der amyotrophen Lateralsklerose
Greek	Ταλαμπανέλη	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Talampanel	Amyotrophiás lateral sclerosis kezelése
Italian	Talampanel	Trattamento della sclerosi laterale amiotrofica
Latvian	Talampanels	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Talampanelis	Šoninės amiotrofinės sklerozės gydymas
Maltese	Talampanel	Kura tas-sklersi laterali amjotrofika
Polish	Talampanel	Leczenie stwardnienia bocznego zanikowego
Portuguese	Talampanel	Tratamento da esclerose lateral amiotrófica
Romanian	Talampanel	Tratamentul sclerozei laterale amiotrofice
Slovak	Talampanel	Liečba amyotrofickéj laterálnej sklerózy
Slovenian	Talampanel	Zdravljenje amiotrofične lateralne skleroze
Spanish	Talampanel	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Talampanel	Behandling av amyotrofisk lateralskleros
Norwegian	Talampanel	Behandling av amyotrofisk lateralsklerose
Icelandic	Talampanel	Meðferð við blandaðri hreyfitaugahrönnun

¹ At the time of designation