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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Xaliproden hydrochloride for the treatment of amyotrophic lateral sclerosis

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in May 2010 on request of the sponsor.

On 17 January 2001, orphan designation (EU/3/00/015) was granted by the European Commission to Sanofi-Synthélabo, France, for xaliproden hydrochloride or the treatment of amyotrophic lateral sclerosis.

The sponsor Sanofi Synthélabo changed name to Sanofi-Aventis in May 2006.

What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis is a progressive disease of the nervous system that is caused by the gradual deterioration of specific nerve cells in the brain and spinal cord that control voluntary movement. The loss of these so-called motor neurons causes the muscles under their control to weaken and waste away, eventually leading to paralysis. Symptoms of amyotrophic lateral sclerosis vary 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. The reason why neurons deteriorate in amyotrophic lateral sclerosis is thought to be that defective (misfolded) protein molecules accumulate in these cells. Amyotrophic lateral sclerosis is chronically debilitating and life-threatening.

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

At the time of designation, amyotrophic lateral sclerosis affected less than 1 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 38,000 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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition.

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What treatments are available?

Riluzole is the only medicinal product authorised in the European Community for the treatment of patients with amyotrophic lateral sclerosis. Xaliproden hydrochloride might be of potential significant benefit for the treatment of amyotrophic lateral sclerosis because it might improve the overall outcome of the patients. 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?

The exact mechanism by which xaliproden hydrochloride works has not been fully characterised, but it is thought to protect the motor neurons from wasting away, and thus improve the symptoms of the patients.

What is the stage of development of this medicine?

The effects of xaliproden hydrochloride were evaluated in experimental models. At the time of submission of the application for orphan designation, three clinical trials in patients with amyotrophic lateral sclerosis were ongoing and eight were completed.

Xaliproden hydrochloride was not marketed anywhere worldwide for the treatment of amyotrophic lateral sclerosis, at the time of submission. Orphan designation of xaliproden hydrochloride was granted in Japan for condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 November 2000 recommending the granting of this designation.

Update: Since the orphan designation of xaliproden hydrochloride, the clinical development in patients with amyotrophic lateral sclerosis has been discontinued. The orphan designation in Japan has been revoked.

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 European Union) 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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Patient associations' contact points

Pending

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Xaliproden hydrochloride	Treatment of amyotrophic lateral sclerosis
Danish	Xaliprodenhydrochlorid	Behandling af amyotrofisk lateralsklerose
Dutch	Xaliprodenhydrochloride	Behandeling van amyotrofe lateraalsclerose
Finnish	Ksaliprodeenihydrokloridi	Amylotrofisen lateraaliskleroosin hoito
French	Chlorhydrate de xaliprodène	Traitement de la sclérose latérale amyotrophique
German	Xaliprodenhydrochlorid	Zur Behandlung der amyotrophischen Lateralsklerose
Greek	Υδροχλωρική ξαλιπροδένη	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Italian	Xaliproden cloridrato	Trattamento della sclerosi laterale amiotrofica
Portuguese	Cloridrato de xaliprodeno	Tratamento da esclerose lateral amiotrófica
Spanish	Xaliprodeno clorhidrato	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Xaliprodenhydroklorid	Behandling av amyotrofisk lateralskleros

[1] At the time of designation