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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Arimoclomol for the treatment of amyotrophic lateral sclerosis

First publication	1 April 2009
Rev.1: transfer of sponsorship	30 April 2012
Rev.2: administrative update	14 October 2013

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.

Withdrawn

On 26 October 2006, orphan designation (EU/3/06/406) was granted by the European Commission to Wainwright Associates Ltd, United Kingdom, for arimoclomol for the treatment of amyotrophic lateral sclerosis.

The sponsorship was transferred to Orphazyme ApS, Denmark, in January 2012.

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

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

At the time of designation, amyotrophic lateral sclerosis affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 28,000 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?

A medicinal product called riluzole was authorised for the condition in the Community at the time of submission of the application for orphan drug designation. The sponsor has provided sufficient information to show that arimoclomol might be of significant benefit for patients with amyotrophic lateral sclerosis.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?

Misfolding of some proteins in motor-neurons is thought to be part of the development of amyotrophic lateral sclerosis. Arimoclomol is a small molecule that stimulates the production of so-called chaperones, which are proteins that help these proteins folding correctly. This is hoped to decrease the loss of motor-neurons, and thus improve the symptoms.

What is the stage of development of this medicine?

The effects of arimoclomol were evaluated in experimental models.

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

Arimoclomol was not authorised anywhere worldwide for the treatment of amyotrophic lateral sclerosis, at the time of submission.

Orphan designation of arimoclomol was granted in the United States for the treatment of this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 September 2006 recommending the granting of this designation.

Withdrawn

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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.

Withdrawn

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

Language	Active ingredient	Indication
English	Arimoclomol	Treatment of amyotrophic lateral sclerosis
Bulgarian	Аримокломол	Лечение на амиотрофична латерална склероза
Czech	Arimoklomol	Léčba amyotrofické laterální sklerózy
Danish	Arimoclomol	Behandling af amyotrofisk lateralsklerose
Dutch	Arimoclomol	Behandeling van amyotrofe lateraalsclerose
Estonian	Arimoklomool	Amüotroofilise lateraalskleroosi ravi
Finnish	Arimoklomoli	Amylotrofisen lateraaliskleroosin hoito
French	Arimoclomol	Traitement de la sclérose latérale amyotrophique
German	Arimoclomol	Behandlung der amyotrophischen Lateralsklerose
Greek	Αριμοκλομόλη	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Arimoclomol	Amyotrophiás lateral sclerosis kezelése
Italian	Arimoclomol	Trattamento della sclerosi laterale amiotrofica
Latvian	Arimoklomols	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Arimoklomolis	Šoninės amiotrofinės sklerozės gydymas
Maltese	Arimoclomol	Kura tas-sklerosi laterali amjotrofika
Polish	Arimoklomol	Leczenie stwardnienia bocznego zanikowego
Portuguese	Arimoclomol	Tratamento da esclerose lateral amiotrófica
Romanian	Arimoclomol	Tratamentul sclerozei laterale amiotrofice
Slovak	Arimoklomol	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Arimoklomol	Zdravljenje amiotrofične lateralne skleroze
Spanish	Arimoclomol	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Arimoklomol	Behandling av amyotrofisk lateralskleros
Norwegian	Arimoklomol	Behandling av amyotrofisk lateralsklerose
Icelandic	Arímóklómól	Meðferð við blandaðri hreyfitaugahrörnun

[1] At the time of transfer of sponsorship