EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sarsasapogenin for the treatment of amyotrophic lateral sclerosis

On 8 May 2008, orphan designation (EU/3/08/543) was granted by the European Commission to Phytopharm plc, United Kingdom for sarsasapogenin for the treatment of amyotrophic lateral sclerosis.

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products on 23 July 2012 on request of the sponsor.

What is amyotrophic lateral sclerosis?

Amyotrophic lateral sclerosis is a progressive disease of the nervous system. Amyotrophic lateral sclerosis occurs when specific nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate. The loss of these so-called motor neurons causes the muscles under their control to weaken and waste away, leading to paralysis. Amyotrophic lateral sclerosis varies 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. Amyotrophic lateral sclerosis strikes in mid-life. Men are about one-and-a-half times more likely to have the disease than women. 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 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 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. Sarsasapogenin might be of potential

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 502,800,000 (Eurostat 2008). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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significant benefit for the treatment of amyotrophic lateral sclerosis. The benefit will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Neurotrophic factors are proteins that improve the survival and function of nerve cells. Although the mechanism of action of sarsasapogenin in amyotrophic lateral sclerosis is not fully understood, it is thought that it increases neurotrophic factors and mimics their effects in the nervous system, and thereby improves the symptoms of the disease.

What is the stage of development of this medicine?

The effects of sarsasapogenin 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 planned.

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

Orphan designation of sarsasapogenin was granted in the United States for the treatment of amyotrophic lateral sclerosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 March 2008 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	Sarsasapogenin	Treatment of amyotrophic lateral sclerosis
Bulgarian	Сарсасапогенин	Амиотрофична латерална склероза
Czech	Sarsasapogenin	Amyotrofická laterální skleróza
Danish	Sarsasapogenin	Amyotrofisk lateral sklerose
Dutch	Sarsasapogenine	Amyotrofe laterale sclerose
English	Sarsasapogenin	Amyotrophic Lateral Sclerosis
Estonian	Sarsasapogeniin	Amüotroofiline lateraalskleroos
Finnish	Sarsasapogeniini	Amyotrofinen lateraaliskleroosi.
French	Sarsapogenine	Sclérose latérale amyotrophique
German	Sarsasapogenin	Amyotrophische Lateralsklerose
Greek	Sarsasapogenin	Αμυοτροφική Πλευρική Σκλήρυνση
Hungarian	Sarsasapogenin	ALS (amiotróf laterális szklerózis)
Icelandic	Sarsasapógenín	Blönduð hreyfitaugahrörnun
Italian	Sarsasapogenina	Sclerosi laterale amiotrofica
Latvian	Sarsasapogenīns	Amiotrofiskā laterālā skleroze
Lithuanian	Sarsasapogeninas	Šoninė amiotrofinė sklerozė
Maltese	Sarsasapogenin	Sklerosi Amjotrofika Laterali
Norwegian	Sarsasapogenin	Amytrofisk lateralsklerose
Polish	Sarsasapogenina	Stwardnienie zanikowe boczne
Portuguese	Sarsasapogenin	Esclerose Lateral Amiotrófica
Romanian	Sarsasapogenină	Scleroză laterală amiotrofică
Slovak	Sarsasapogenín	Amyotrofická laterálna skleróza
Slovenian	Sarsasapogenin	Amiotrofna lateralna skleroza
Spanish	Sarsasapogenina	Esclerosis lateral amiotrófica
Swedish	Sarsasapogenin	Amyotrofisk lateralskleros
Norwegian	Sarsasapogenin	Behandling av amyotrofisk lateralsklerose
Icelandic	Sarsasapógenín	Meðferð við blandaðri hreyfitaugahrörnun

[1] At the time of designation