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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Smilagenin for the treatment of amyotrophic lateral sclerosis

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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.

On 27 September 2011, orphan designation (EU/3/11/914) was granted by the European Commission to Phytopharm plc, United Kingdom, for smilagenin for the treatment of amyotrophic lateral sclerosis.

The sponsorship was transferred to QRC Consultants Ltd., United Kingdom, in October 2013.

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. This causes 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, 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 debilitating and life-threatening disease because of the gradual loss of muscle 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 affected by the condition?

At the time of designation, ALS affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 people[*], and is below the ceiling for orphan designation,

[*]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.
At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

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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, one medicine called 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. The sponsor has provided sufficient information to show that smilagenin might be of significant benefit for patients with ALS because it works in a different way to the existing treatment and early studies show that it might improve the outcome of patients with this condition. These assumptions 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 precise cause of the damage to nerves cells in ALS is unknown but it is thought that a possible cause could be the impairment of neurotrophic factors. Neurotrophic factors are proteins released by a tissue in the body to support the survival and development of nerves in that tissue.

Smilagenin is expected to work by stimulating the production of neurotrophic factors, thereby reducing nerve damage and improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of smilagenin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with ALS had started.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of ALS or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 July 2011 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	Smilagenin	Treatment of amyotrophic lateral sclerosis
Bulgarian	Смилагенин	Лечение на амиотрофична латерална склероза
Croatian	Smilagenin	Liječenje amiotrofične lateralne skleroze
Czech	Smilagenin	Léčba amyotrofické laterální sklerózy
Danish	Smilagenin	Behandling af amyotrofisk lateralsklerose
Dutch	Smilagenine	Behandeling van amyotrofe lateraalsclerose
Estonian	Smilageniin	Amüotroofilise lateraalskleroosi ravi
Finnish	Smilageniini	Amyotrofisen lateraaliskleroosin hoito
French	Smilagénine	Traitement de la sclérose latérale amyotrophique
German	Smilagenin	Behandlung der amyotrophen Lateralsklerose
Greek	Σμιλαγενίνη	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Smilagenin	Amyotrophiás lateral sclerosis kezelése
Italian	Smilagenin	Trattamento della sclerosi laterale amiotrofica
Latvian	Smilagenīns	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Smilageninas	Šoninės amiotrofinės sklerozės gydymas
Maltese	Smilagenin	Kura tas-sklerosi laterali amjotrofika
Polish	Smilagenina	Leczenie stwardnienia bocznego zanikowego
Portuguese	Smilagenina	Tratamento da esclerose lateral amiotrófica
Romanian	Smilageninǎ	Tratamentul sclerozei laterale amiotrofice
Slovak	Smilagenín	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Smilagenin	Zdravljenje amiotrofične lateralne skleroze
Spanish	Smilagenina	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Smilagenin	Behandling av amyotrofisk lateralskleros
Norwegian	Smilagenin	Behandling av amyotrofisk lateralsklerose
Icelandic	Smílagenín	Meðferð við blandaðri hreyfitaugahrörnun

[1] At the time of transfer of sponsorship