20 June 2016
EMA/COMP/310646/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate for the treatment of amyotrophic lateral sclerosis

On 30 May 2016, orphan designation (EU/3/16/1662) was granted by the European Commission to QRC Consultants Ltd, United Kingdom, for H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate (also known as GM604) 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 nerve cells in the brain and spinal cord that control voluntary movement gradually deteriorate, causing loss of muscle function and paralysis. The exact causes are unknown but are believed to include genetic and environmental factors. The symptoms of ALS depend on which muscles weaken first, and include loss of balance, loss of control of hand and arm movement, and 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 long-term debilitating and life-threatening disease because of the gradual loss of function and its paralysing effect on muscles used for breathing which usually leads to death from respiratory failure.

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 was equivalent to a total of around 51,000 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).

[*]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 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

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What treatments are available?

At the time of designation, riluzole was authorised in the EU to treat ALS. Patients also received supportive treatment to relieve the symptoms of the disease, such as physiotherapy and speech therapy.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with ALS because laboratory studies as well as early studies in ALS patients show that the medicine might improve symptoms such as breathing, muscle function and muscle strength. 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 medicine, H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate, is made of 6 amino acids. The amino acids are arranged in the same order as those in a part of MTNF, a substance which regulates the development of the nervous system in a fetus. Like MTNF, the medicine is expected to act on genes that help to regenerate and repair nerve cells. In this way, the medicine is expected to help reduce damage to nerve cells caused by ALS.

What is the stage of development of this medicine?

The effects of H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 April 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate	Treatment of amyotrophic lateral sclerosis
Bulgarian	Н-Фен-Сер-Арг-Тир-Ала-Арг-ОН acetate	Лечение на амиотрофична латерална склероза
Croatian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetat	Liječenje amiotrofične lateralne skleroze
Czech	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate	Léčba amyotrofické laterální sklerózy (ALS)
Danish	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetat	Behandling af amyotrofisk lateralsklerose
Dutch	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate	Behandeling van amyotrofe lateraalsclerose
Estonian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH atsetaat	Amüotroofilise lateraalskleroosi ravi
Finnish	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH asetaatti	Amyotrofisen lateraaliskleroosin hoito
French	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acétate	Traitement de la sclérose latérale amyotrophique
German	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH Acetat	Behandlung der amyotrophen Lateralsklerose
Greek	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH οξικό	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetát	Amyotrophiás lateral sclerosis kezelése
Italian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetato	Trattamento della sclerosi laterale amiotrofica
Latvian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetāts	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetatas	Šoninės amiotrofinės sklerozės gydymas
Maltese	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate	Kura tas-sklerosi laterali amjotrofika
Polish	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH octan	Leczenie stwardnienia bocznego zanikowego
Portuguese	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetato	Tratamento da esclerose lateral amiotrófica
Romanian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetat	Tratamentul sclerozei laterale amiotrofice
Slovak	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetát	Liečba amyotrofickej laterálnej sklerózy
Slovenian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetate	Zdravljenje amiotrofične lateralne skleroze
Spanish	Acetato de H-Fe-Ser-Arg-Tyr-Ala-Arg-OH-	Tratamiento de la esclerosis lateral amiotrófica
Swedish	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetat	Behandling av amyotrofisk lateralskleros
Norwegian	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetat	Behandling av amyotrofisk lateralsklerose
Icelandic	H-Phe-Ser-Arg-Tyr-Ala-Arg-OH acetat	Meðferð við blandaðri hreyfitaugahrörnun

[1] At the time of designation