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Public summary of opinion on orphan designation

Reldesmetiv for the treatment of amyotrophic lateral sclerosis

On 28 February 2020, orphan designation EU/3/20/2256 was granted by the European Commission to Pharma Gateway AB, Sweden, for reldesemtiv 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 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, amyotrophic lateral sclerosis affected approximately 1.00 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 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?

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

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with ALS. Early studies showed that the medicine could lead to a slower decline of

*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 519,200,000 (Eurostat 2020).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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patients' movements. 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?

This medicine is expected to improve the way that voluntary muscles work by stimulating the action of proteins known as troponins, which are involved in muscle contractions. By doing so, the medicine is expected to strengthen the response of muscles to nerve signals in patients with amyotrophic lateral sclerosis. This is expected to improve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of reldesemtiv have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 22 January 2020, recommending the granting of this designation.

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.

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	Reldesemtiv	Treatment of amyotrophic lateral sclerosis
Bulgarian	Релдесемтив	Лечение на амиотрофична латерална склероза
Croatian	Reldesemtiv	Liječenje amiotrofične lateralne skleroze
Czech	Reldesemtiv	Léčba amyotrofické laterální sklerózy (ALS)
Danish	Reldesemtiv	Behandling af amyotrofisk lateralsklerose
Dutch	Reldesemtiv	Behandeling van amyotrofe lateraalsclerose
Estonian	Reldesemtiiv	Amüotroofilise lateraalskleroosi ravi
Finnish	Reldesemtiivi	Amyotrofisen lateraaliskleroosin hoito
French	Reldésemtive	Traitement de la sclérose latérale amyotrophique
German	Reldesemtiv	Behandlung der amyotrophen Lateralsklerose
Greek	Ρελντεσεμτίβη	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Reldesemtiv	Amyotrophiás lateral sclerosis kezelése
Italian	Reldesemtiv	Trattamento della sclerosi laterale amiotrofica
Latvian	Reldesemtivs	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Reldesemtivas	Šoninės amiotrofinės sklerozės gydymas
Maltese	Reldesemtiv	Kura tas-sklerosi laterali amjotrofika
Polish	Reldesemtiw	Leczenie stwardnienia bocznego zanikowego
Portuguese	Reldesemtiv	Tratamento da esclerose lateral amiotrófica
Romanian	Reldesemtiv	Tratamentul sclerozei laterale amiotrofice
Slovak	Reldesemtiv	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Reldesemtiv	Zdravljenje amiotrofične lateralne skleroze
Spanish	Reldesemtiv	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Reldesemtiv	Behandling av amyotrofisk lateralskleros
Norwegian	Reldesemtiv	Behandling av amyotrofisk lateralsklerose
Icelandic	Reldesemtív	Meðferð við blandaðri hreyfitaugahrörnun

[1] At the time of designation