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# Public summary of opinion on orphan designation

## Recombinant human antibody directed against misfolded human superoxide dismutase 1 for the treatment of amyotrophic lateral sclerosis

On 17 July 2017, orphan designation (EU/3/17/1894) was granted by the European Commission to The Medical & Regulatory Partnership Limited, United Kingdom, for recombinant human antibody directed against misfolded human superoxide dismutase 1 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?

At the time of designation, amyotrophic lateral sclerosis affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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 to relieve the symptoms of the disease, such as physiotherapy and breathing support.

*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 515,700,000 (Eurostat 2017).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with ALS. Laboratory studies showed that the medicine may delay problems with movement and body weight loss, effects which have not been seen with the currently authorised product. 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?

In some patients with ALS, the condition is caused by a mutation (change) in a gene responsible for producing the enzyme SOD1. This mutation leads to the production of a defective SOD1 which is toxic to nerve cells, eventually causing them to die. This medicine is made of an antibody that attaches to defective SOD1 thereby inactivating it. By reducing the amount of defective SOD1, the medicine is expected to improve the symptoms of ALS.

## What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with ALS had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for 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 June 2017 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 | Recombinant human antibody directed against misfolded human superoxide dismutase 1 | Treatment of amyotrophic lateral sclerosis |
| Bulgarian | Рекомбинантно човешко антитяло, насочено срещу човешка неправилно нагъната супероксид дисмутаза 1 | Лечение на амиотрофична латерална склероза |
| Croatian | Rekombinantno humano antitijelo koje djeluje na humanu nepravilno smotanu superoksid-dismutazu 1 | Liječenje amiotrofične lateralne skleroze |
| Czech | Rekombinantní lidská protilátka proti lidské superoxid-dismutáze 1 s defektní konformací | Léčba amyotrofické laterální sklerózy (ALS) |
| Danish | Rekombinante humane antistoffer rettet mod humant fejlfoldet superoxiddismutase 1 | Behandling af amyotrofisk lateralsklerose |
| Dutch | Recombinant humaan antilichaam dat gericht is tegen misvouwd humaan superoxide dismutase 1 | Behandeling van amyotrofe lateraalsclerose |
| Estonian | Rekombinantne inimese antikeha on suunatud inimese valesti kokkupakitud superoksiidi dismutaasi 1 vastu | Amüotroofilise lateraalskleroosi ravi |
| Finnish | Rekombinantti ihmisen vasta-aine väärin laskostunutta superoksididismutaasia 1:tä vastaan | Amyotrofisen lateraaliskleroosin hoito |
| French | Anticorps recombinants humains dirigés contre la superoxyde dismutase 1 humaine mal repliée | Traitement de la sclérose latérale amyotrophique |
| German | Rekombinanter humaner Antikörper, der sich gegen fehlgefaltete humane Superoxiddismutase 1 richtet | Behandlung der amyotrophen Lateralsklerose |
| Greek | Ανασυνδυασμένο ανθρώπινο αντίσωμα κατά της λανθασμένα αναδιπλωμένης ανθρώπινης υπεροξειδικής δισμουτάσης 1 | Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης |
| Hungarian | Emberi nem megfelelően feltekeredett szuperoxid diszmutáz elleni rekombináns emberi antitest 1 | Amyotrophiás lateral sclerosis kezelése |
| Italian | Anticorpi umani ricombinanti diretti contro la superossido dismutasi 1 umana mal ripiegata | Trattamento della sclerosi laterale amiotrofica |
| Latvian | Rekombinanta cilvēka antiviela, kas ir vērsta pret cilvēka kļūdaini salocītu superoksīda 1. dismutāzi | Amiotrofiskās laterālās sklerozes ārstēšana |
| Lithuanian | Rekombinantinis žmogaus antikūnas, nukreiptas prieš netinkamai susilanksčiusią žmogaus superoksido dismutazę 1 | Šoninės amiotrofinės sklerozės gydymas |
| Maltese | Antikorp uman rikombinanti dirett kontra superoxide dismutase 1 mitwi b'mod inkorrett | Kura tas-sklerosi laterali amjotrofika |
| Polish | Rekombinowane przeciwciało ludzkie skierowane przeciwko źle sfałdowanej dyzmutazie ponadtlenkowej 1 | Leczenie stwardnienia bocznego zanikowego |
| Portuguese | Anticorpo humano recombinante dirigido contra a superóxido dismutase 1 humana com uma conformação espacial deficiente | Tratamento da esclerose lateral amiotrófica |

[1] At the time of designation

| Language | Active ingredient | Indication |
|---|---|---|
| Romanian | Anticorp recombinant uman direcţionat împotriva superoxid dismutazei 1 umane greşit pliate | Tratamentul sclerozei laterale amiotrofice |
| Slovak | Rekombinantná ľudská protilátka určená proti patologickej konformácii superoxid dismutázy 1 | Liečba amyotrofickej laterálnej sklerózy |
| Slovenian | Rekombinantno človeško protitelo, usmerjeno proti nepravilno zviti človeški superoksid dismutazi 1 | Zdravljenje amiotrofične lateralne skleroze |
| Spanish | Anticuerpo humanizado recombinante dirigido contra la superóxido dismutasa 1 humana mal plegada | Tratamiento de la esclerosis lateral amiotrófica |
| Swedish | Rekombinant human antikropp riktad mot human felveckad superoxiddismutas 1 | Behandling av amyotrofisk lateralskleros |
| Norwegian | Rekombinant humant antistoff rettet mot humant misfoldet superoksiddismutase 1 | Behandling av amyotrofisk lateralsklerose |
| Icelandic | Manna raðbrigða mótefni úr sem beinist gegn misfelldum manna-súperoxíðdismútasa 1 | Meðferð við blandaðri hreyfitaugahrörnun |