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EMA/COMP/510068/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid for the treatment of amyotrophic lateral sclerosis

On 29 August 2016, orphan designation (EU/3/16/1732) was granted by the European Commission to Biogen Idec Limited, United Kingdom, for synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid (also known as BIIB067) 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, 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 breathing support.

The sponsor has provided sufficient information to show that medicine might be of significant benefit for patients with ALS, with laboratory studies showing improvement in movements not 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 a small strand of synthetic genetic material that blocks the production of defective SOD1. By reducing the amount of defective SOD1, this medicine is expected to improve the symptoms of ALS.

What is the stage of development of this medicine?

The effects of the medicine 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 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 13 July 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	Synthetic ribonucleic acid oligonucleotide directed against superoxide dismutase 1 messenger ribonucleic acid	Treatment of amyotrophic lateral sclerosis
Bulgarian	Синтетичен олигонуклеотид от рибонуклеинова киселина, насочен срещу иРНК на супероксид дисмутаза 1	Лечение на амиотрофична латерална склероза
Croatian	Sintetička oligonukleotidna ribonukleinska kiselina usmjerena protiv ribonukleinske kiseline glasnika superoksid dismutaze 1	Liječenje amiotrofične lateralne skleroze
Czech	Syntetický oligonukleotid ribonukleové kyseliny namířený proti superoxiddismutázy 1 messenger ribonukleové kyseliny	Léčba amyotrofické laterální sklerózy (ALS)
Danish	Syntetisk ribonukleinsyre oligonukleotid rettet mod superoxiddismutase 1 messenger ribonukleinsyre	Behandling af amyotrofisk lateralsklerose
Dutch	Synthetische ribonucleïnezuur oligonucleotide gericht tegen superoxidedismutase 1 messenger ribonucleïnezuur	Behandeling van amyotrofe lateraalsclerose
Estonian	Sünteetiline ribonukleiinhappe oligonukleotiid, mis on suunatud superoksiidi dismutaas 1 messenger ribonukleiinhappe vastu	Amüotroofilise lateraalskleroosi ravi
Finnish	Synteettinen ribonukleiinihappo oligonukleotidi, joka on suunnattu superoksididismutaasi 1 lähetti-ribonukleiinihappoa vastaan	Amyotrofisen lateraaliskleroosin hoito
French	Oligonucléotide synthétique d'acide ribonucléique dirigé contre de l'acide ribonucléique messager de la superoxyde dismutase 1	Traitement de la sclérose latérale amyotrophique
German	Synthetische Ribonukleinsäure Oligonukleotid gegen Superoxid-Dismutase 1 Boten-Ribonukleinsäure	Behandlung der amyotrophen Lateralsklerose
Greek	Συνθετικό ολιγονουκλεοτίδιο ριβονουκλεϊκού οξέως που στρέφεται εναντίον της υπεροξειδικής δισμουτάσης 1 αγγελιοφόρου ριβονουκλεϊκού οξέως	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Szuperoxid dizmutáz 1 hírvivő ribonukleinsav-ellenes szintetikus ribonukleinsav oligonukleotid	Amyotrophiás lateral sclerosis kezelése
Italian	Oligonucleotide sintetico acido ribonucleico diretto contro l' acido ribonucleico messaggero della superossido dismutasi 1	Trattamento della sclerosi laterale amiotrofica
Latvian	Sintētisks ribonukleīnskābes oligonukleotīds, kas vērsts pret superoksīda dismutāzes 1 mesendžera ribonukleīnskābi	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Sintetinis ribonukleininės rūgšties oligonukleotidas, nukreiptas prieš superoksido dismutazės 1 informacinę ribonukleininę rūgštį	Šoninės amiotrofinės sklerozės gydymas

[1] At the time of designation

Language	Active ingredient	Indication
Maltese	Oligonukleotide ta' ribonucleic acid sintetiku dirett kontra superoxide dismutase 1 messenger ribonucleic acid	Kura tas-sklerosi laterali amjotrofika
Polish	Syntetyczny oligonukleotyd kwasu rybonukleinowego skierowany przeciwko matrycowemu kwasowi rybonukleinowemu dysmutazy ponadtlenkowej 1	Leczenie stwardnienia bocznego zanikowego
Portuguese	Oligonucleótido sintético de ácidos ribonucleicos dirigido contra o ácido ribonucleico mensageiro da superóxido dismutase 1	Tratamento da esclerose lateral amiotrófica
Romanian	Oligonucleotidă sintetică de acid ribonucleic îndreptată împotriva acidului ribonucleic mesager al superoxid dismutazei 1	Tratamentul sclerozei laterale amiotrofice
Slovak	Syntetický oligonukleotid ribonukleovej kyseliny namierený proti messengerovej ribonukleovej kyseline superoxiddismutázy 1	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Sintetični oligonukleotid ribonukleinske kisline usmerjen proti superoksidni dismutazi 1 messenger ribonukleinske kisline	Zdravljenje amiotrofične lateralne skleroze
Spanish	oligonucleótido ácido ribonucleico sintético dirigido contra la superóxido 1 ácido ribonucleico mensajero superóxido	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Syntetisk ribonukleinsyra oligonukleotid riktad mot superoxiddismutas 1 budbärare ribonukleinsyra	Behandling av amyotrofisk lateralskleros
Norwegian	Syntetisk ribonukleinsyre oligonukleotid rettet mot superoksid dismutase 1 budbringer ribonukleinsyre	Behandling av amyotrofisk lateralsklerose
Icelandic	Tilbúið ríbósakjarnsýra fánúkleótíð beint gegn súperoxíð dismútasa 1 boðberaríbósakjarnsýra	Meðferð við blandaðri hreyfitaugahrörnun