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Public summary of opinion on orphan designation

Dextran sulfate low molecular weight for the treatment of amyotrophic lateral sclerosis

On 21 August 2020, orphan designation EU/3/20/2318 was granted by the European Commission to TikoMed AB, Sweden, for dextran sulfate low molecular weight (also known as ILB) 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 midlife 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 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.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with amyotrophic lateral sclerosis because early data suggest that the medicine could improve patients' physical function and symptoms.

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 way the medicine might work in ALS is not fully understood. The medicine is expected to work by increasing the release and activation of growth factors, molecules in the body that stimulate cell growth. This is thought to help regenerate damaged tissues and reduce nerve cell death, thereby improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of dextran sulfate low molecular weight have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with dextran sulfate low molecular weight in patients with amyotrophic lateral sclerosis were ongoing.

At the time of submission, the medicine 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 16 July 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

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	Dextran sulfate low molecular weight	Treatment of amyotrophic lateral sclerosis
Bulgarian	Нискомолекулярен декстран сулфат	Лечение на амиотрофична латерална склероза
Croatian	Dekstran sulfat niske molekularne mase	Liječenje amiotrofične lateralne skleroze
Czech	Nízkomolekulární dextran sulfát	Léčba amyotrofické laterální sklerózy (ALS)
Danish	Dextransulfat, lavmolekylær	Behandling af amyotrofisk lateralsklerose
Dutch	Dextraansulfaat met laag moleculair gewicht	Behandeling van amyotrofe lateraalsclerose
Estonian	Madalmolekulaarne dekstraan-sulfaat	Amüotroofilise lateraalskleroosi ravi
Finnish	Dekstraanisulfaatti pienimolekyylipainoinen	Amyotrofisen lateraaliskleroosin hoito
French	Sulfate de dextrane de bas poids moléculaire	Traitement de la sclérose latérale amyotrophique
German	Dextransulfat mit niedrigem Molekulargewicht	Behandlung der amyotrophen Lateralsklerose
Greek	Θειική δεξτράνη χαμηλού μοριακού βάρους	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Alacsony molekulasúlyú dextran szulfát	Amyotrophiás lateral sclerosis kezelése
Italian	Destrano solfato a basso peso molecolare	Trattamento della sclerosi laterale amiotrofica
Latvian	Zemas molekulārās masas dekstrāna sulfāts	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Mažos molekulinės masės dekstrano sulfatas	Šoninės amiotrofinės sklerozės gydymas
Maltese	Piż molekulari baxx tas-sulfat dežtran	Kura tas-sklerosi laterali amjotrofika
Polish	Siarczan dekstranu o niskiej wadze cząsteczkowej	Leczenie stwardnienia bocznego zanikowego
Portuguese	Sulfato de dextrano de baixo peso molecular	Tratamento da esclerose lateral amiotrófica
Romanian	Dextran sulfat cu greutate moleculară mică	Tratamentul sclerozei laterale amiotrofice
Slovak	Dextrán sulfát nízka molekulárna hmotnosť	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Nizkomolekularni dekstransulfat	Zdravljenje amiotrofične lateralne skleroze
Spanish	sulfato de dextrano de bajo peso molecular	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Dextransulfat låg molekylär vikt	Behandling av amyotrofisk lateralskleros
Norwegian	Dekstransulfat med lav molekylvekt	Behandling av amyotrofisk lateralsklerose

[1] At the time of designation

Language	Active ingredient	Indication
Icelandic	Dextransúlfat með lítinn sameindamassa	Meðferð við blandaðri hreyfitaugahrörnun