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Public summary of opinion on orphan designation

Human culture expanded autologous mesenchymal stromal cells for the treatment of amyotrophic lateral sclerosis

On 24 April 2019, orphan designation (EU/3/19/2155) was granted by the European Commission to IQVIA RDS Ireland Limited, Ireland, for human culture expanded autologous mesenchymal stromal cells (also known as lenzumestrocel) 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 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. Baclofen was authorised for treating muscle symptoms which include those of 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 518,400,000 (Eurostat 2019).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with ALS because preliminary results show that adding it to riluzole slowed progression of the condition. 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 contains cells called mesenchymal stromal cells (MSCs) obtained from the patient's bone marrow and grown in a laboratory to increase their numbers. MSCs are thought to increase production of substances that prevent inflammation, protect nerve cells and reduce the activity of the immune system (the body's natural defences). Injecting the medicine into the patient's spine is expected to prevent the immune system from damaging nerve cells and so prevent worsening of the condition.

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.

The medicine is authorised in South Korea for the treatment of ALS.

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 21 March 2019, 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	Human culture expanded autologous mesenchymal stromal cells	Treatment of amyotrophic lateral sclerosis
Bulgarian	Култивирани човешки автоложни мезенхимни стромални клетки	Лечение на амиотрофична латерална склероза
Croatian	Ljudske autologne mezenhimalne stromalne stanice umnožene u kulturi	Liječenje amiotrofične lateralne skleroze
Czech	Lidské expandované autologní mesenchymální stromální buňky	Léčba amyotrofické laterální sklerózy (ALS)
Danish	Humane kultur expanderede autologe mesenkymale stomale celler	Behandling af amyotrofisk lateralsklerose
Dutch	Uit humane (cel)cultuur geëxpandeerde autologe mesenchymale stromale cellen	Behandeling van amyotrofe lateraalsclerose
Estonian	Inimese autoloogsed mesenhümaalsed kultuuris paljundatud stroomarakud	Amüotroofilise lateraalskleroosi ravi
Finnish	Ihmisen kasvattamalla laajennetut, autologiset mesenkymaaliset stroomasolut	Amyotrofisen lateraaliskleroosin hoito
French	Cellules stromales mésenchymateuses autologues humaines amplifiées par culture	Traitement de la sclérose latérale amyotrophique
German	Humane kultivierte expandierte autologe mesenchymale Stromazellen	Behandlung der amyotrophen Lateralsklerose
Greek	Ανθρώπινα καλλιεργημένα αυτόλογα μεσεγχυματικά στρωματικά κύτταρα	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Sejttenyésztéssel szaporított autológ human mesenchimális stroma sejtek	Amyotrophiás lateral sclerosis kezelése
Italian	Cellule stromali mesenchimali autologhe umane espanse in coltura	Trattamento della sclerosi laterale amiotrofica
Latvian	Kultūrā pavairotas autologas cilvēka mezenhimālās stromas šūnas	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Žmogaus kultūra išskirta iš autologinių mezenchimos stromos ląstelių	Šoninės amiotrofinės sklerozės gydymas
Maltese	Ċelloli stromali mesenkimali awtologi espanduti tal-kultura tal-bniedem	Kura tas-sklerosi laterali amjotrofika
Polish	Ludzkie autologiczne mezenchymalne komórki zrębu namnożone w kulturze komórkowej	Leczenie stwardnienia bocznego zanikowego
Portuguese	Células mesenquimais estromais autólogas humanas expandidas em cultura	Tratamento da esclerose lateral amiotrófica

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Celule stromale mezenchimale autologe umane expandate în cultură	Tratamentul sclerozei laterale amiotrofice
Slovak	Autológne mezenchymálne stromálne bunky expandované z ľudskej kultúry	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Humane, v kulturi ekspandirane, avtologne mezenshimske stromalne celice	Zdravljenje amiotrofične lateralne skleroze
Spanish	Celulas humanas mesenquimales estroma autologas cultivadas y expandidas	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Humana odlade expanderade autologa mesenkymala stromalceller	Behandling av amyotrofisk lateralskleros
Norwegian	Humane autologe mesenkymale stromale celler ekspandert i kultur	Behandling av amyotrofisk lateralsklerose
Icelandic	Samgena, manna, bandvefsgrunnfrumur sem látnar eru fjölga sér í ræktun.	Meðferð við blandaðri hreyfitaugahrörnun