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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Allogeneic motor neuron progenitor cells derived from human embryonic stem cells for the treatment of amyotrophic lateral sclerosis

On 17 July 2013, orphan designation (EU/3/13/1155) was granted by the European Commission to California Stem Cell (UK) Ltd, United Kingdom, for allogeneic motor neuron progenitor cells derived from human embryonic stem cells 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. This causes loss of muscle function and paralysis. The exact causes are unknown but are believed to include genetic and environmental factors. The symptoms of ALS vary depending on which muscles weaken first, and include loss of balance, loss of control of hand and arm movement, 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 long-term 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 due to respiratory failure.

What is the estimated number of patients affected by the condition?

At the time of designation, ALS affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 25,000 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 27), Norway, Iceland and Liechtenstein. This represents a population of 509,000,000 (Eurostat 2013).

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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 temporarily relieve the symptoms of the disease, such as physiotherapy and speech therapy.

The sponsor has provided sufficient information to show that the medicine containing allogeneic motor neuron progenitor cells derived from human embryonic stem cells might be of significant benefit for patients with ALS because it works in a different way to currently authorised treatment, and early studies in preclinical models show that it might improve the treatment of patients with this condition when used alone or in combination with riluzole. These assumptions 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 consists of motor neuron progenitor cells (cells that develop into motor neurons). These have been derived from human embryonic stem cells, immature cells in the embryo that are capable of developing into different kinds of cells.

The medicine is intended for injection into the spinal cord of patients with ALS. Once inside the spinal cord, the cells are expected to develop into mature motor neurons which will help to improve the patient's condition by releasing certain natural substances (known as growth factors) that delay the loss of motor-neuron function and possibly by replacing some of the patient's poorly functioning or lost motor neurons.

What is the stage of development of this medicine?

The effects of allogeneic motor neuron progenitor cells derived from human embryonic stem cells have been evaluated in experimental models.

At the time of submission of the application for orphan designation, 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 13 June 2013 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:

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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	Allogeneic motor neuron progenitor cells derived from human embryonic stem cells	Treatment of amyotrophic lateral sclerosis
Bulgarian	Алогенни прогениторни клетки на моторни неврони, получени от човешки ембрионални стволови клетки	Лечение на амиотрофична латерална склероза
Czech	Alogenní progenitorové buňky motorických neuronů odvozené z lidských embryonálních kmenových buněk	Léčba amyotrofické laterální sklerózy
Croatian	Alogene progenitorske stanice motoričkih neurona dobivene iz matičnih stanica ljudskog embrija	Liječenje amiotrofične lateralne skleroze
Danish	Allogene motorneuron-progenitorceller deriveret fra humane embryoniske stamceller	Behandling af amyotrofisk lateralsklerose
Dutch	Allogene motorneuron progenitorcellen afgeleid uit humane embryonale stamcellen	Behandeling van amyotrofe lateraalsclerose
Estonian	Inimembrüo tüvirakkudest saadud allogeensed motoneuroni eellasrakud	Amüotroofilise lateraalskleroosi ravi
Finnish	Allogeenisia motoneuronien progenitorisoluja, jotka ovat peräisin ihmisalkion kantasoluista	Amyotrofisen lateraaliskleroosin hoito
French	Cellules progénitrices de neurones moteurs allogéniques dérivées de cellules souches embryonnaires humaines	Traitement de la sclérose latérale amyotrophique
German	Allogene Motorneuron-Vorläuferzellen,, abgeleitet von menschlichen embryonalen Stammzellen	Behandlung der amyotrophen Lateralsklerose
Greek	Αλλογενή προγονικά κύτταρα κινητικών νευρώνων που προέρχονται από ανθρώπινα εμβρυϊκά βλαστικά κύτταρα	Θεραπεία πλάγιας μυοατροφικής σκλήρυνσης
Hungarian	Humán embrionális őssejtből származó allogén motoros neuron progenitor sejtek	Amyotrophiás lateral sclerosis kezelése
Italian	Cellule progenitrici motoneuronali allogeniche derivate da cellule staminali embrionali umane	Trattamento della sclerosi laterale amiotrofica
Latvian	Allogēnas motoneirona priekšgājēju šūnas, kas atvasinātas no cilvēka embrija cilmes šūnām	Amiotrofiskās laterālās sklerozes ārstēšana
Lithuanian	Alogeninio motorinio neurono pirmtako ląstelės, išskirtos iš žmogaus embrioninių kamieninių ląstelių	Šoninės amiotrofinės sklerozės gydymas
Maltese	Ċelluli proġenituri alloġeneiċi tal- motor newron imnissla minn celluli staminali embrjoniċi umani	Kura tas-sklerosi laterali amjotrofika
Polish	Allogeniczne komórki progenitorowe neuronu ruchowego pochodzące z komórek macierzystych zarodków ludzkich	Leczenie stwardnienia bocznego zanikowego

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	Células progenitoras alogénicas do neurónio motor derivadas de células estaminais embrionárias humanas	Tratamento da esclerose lateral amiotrófica
Romanian	Celule alogeneice progenitoare de neuron motor derivate din celule stem embrionare umane	Tratamentul sclerozei laterale amiotrofice
Slovak	Alogénne progenitorové bunky motorických neurónov derivované z ľudských embryonálnych kmeňových buniek	Liečba amyotrofickej laterálnej sklerózy
Slovenian	Alogenične progenitorne celice motoričnih nevronov, pridobljene iz embrionalnih matičnih celic	Zdravljenje amiotrofične lateralne skleroze
Spanish	Células alogénicas progenitoras de neuronas motoras procedentes de células madre embrionarias humanas	Tratamiento de la esclerosis lateral amiotrófica
Swedish	Allogena motorneuronprogenitorceller utvunna ur mänskliga embryonala stamceller	Behandling av amyotrofisk lateralskleros
Norwegian	Allogene motornevron progenitorceller avledet fra humane embryonale stamceller	Behandling av amyotrofisk lateralsklerose
Icelandic	Ósamgena hreyfitaugunga stofnfrumur fengnar frá stofnfrumum úr fósturvísum manna	Meðferð við blandaðri hreyfitaugahrörnun