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EMA/COMP/2194/2013
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 5q spinal muscular atrophy

On 24 January 2013, orphan designation (EU/3/12/1088) 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 5q spinal muscular atrophy.

What is 5q spinal muscular atrophy?

5q spinal muscular atrophy is an inherited disease that affects the motor neurons (nerves from the brain and spinal cord that control muscle movements). Patients with the disease lack a protein called 'survival motor neuron' (SMN), which is essential for the normal functioning and survival of motor neurons. Without this protein, the motor neurons deteriorate and eventually die. This causes the muscles to fall into disuse, leading to muscle wasting (atrophy) and weakness. The disease is linked to a defect on chromosome 5q and is usually diagnosed in the first year of life.

5q spinal muscular atrophy is a long-term debilitating and life-threatening disease because it causes breathing problems and paralysis that worsens over time.

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

At the time of designation, 5q spinal muscular atrophy affected less than 0.4 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 20,000 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, no satisfactory methods were authorised in the EU for the treatment of 5q spinal muscular atrophy. Patients received supportive treatment to help them and their families

*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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cope with the symptoms of the disease. This included chest physiotherapy and physical aids to support muscular function, and ventilators to help with breathing.

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 infants with 5q spinal muscular atrophy. Once inside the spinal cord, the cells are expected to develop into mature motor neurons which will help to improve the child'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 child's poorly functioning or lost motor neurons.

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 of the application for orphan designation, no clinical trials with the medicine in patients with 5q spinal muscular atrophy had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for 5q spinal muscular atrophy. Orphan designation had been granted in the United States of America for 5q spinal muscular atrophy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 December 2012 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 5q spinal muscular atrophy
Bulgarian	Алогенни прогениторни клетки на моторни неврони, получени от човешки ембрионални стволови клетки	Лечение на 5q спинална мускулна атрофия
Czech	Alogenní progenitorové buňky motorických neuronů odvozené z lidských embryonálních kmenových buněk	Léčba 5q spinální muskulární atrofie
Danish	Allogene motorneuron-progenitorceller deriveret fra humane embryonale stamceller	Behandling af 5q spinal muskelatrofi
Dutch	Allogene progenitorcellen van motorneuronen afgeleid van humane embryonale stamcellen	Behandeling van 5q spinale spieratrofie
Estonian	Inimembrüo tüvirakkudest saadud allogeensed motoneuroni eellasrakud	5q spinaalse lihasatroofia ravi
Finnish	Allogeenisia motoneuronien progenitorisoluja, jotka ovat peräisin ihmisalkion kantasoluista	Selkärangan 5q lihassurkastuman hoito
French	Cellules progénitrices de neurones moteurs allogéniques dérivées de cellules souches embryonnaires humaines	Traitement de l'amyotrophie spinale 5q
German	Allogene Progenitorzellen für Motorneurone, abgeleitet von menschlichen embryonalen Stammzellen	Behandlung der 5q spinalen Muskelatrophie
Greek	Αλλογενή προγονικά κύτταρα κινητικών νευρώνων που προέρχονται από ανθρώπινα εμβρυϊκά βλαστικά κύτταρα	Θεραπεία της νωτιαίας μυϊκής ατροφίας (5q)
Hungarian	Humán embrionális őssejtből származó allogén motoros neuron progenitor sejtek	5q spinális izomatrophia kezelése
Italian	Cellule progenitrici motoneuronali allogeniche derivate da cellule staminali embrionali umane	Trattamento dell'atrofia muscolare spinale 5q
Latvian	Allogēnas motoneirona priekšgājēju šūnas, kas atvasinātas no cilvēka embrija šūnām	5q spinālas muskuļu atrofijas ārstēšana
Lithuanian	Alogeninio motorinio neurono pirmtako ląstelės, gautos iš žmogaus embrioninių kamieninių ląstelių	Nugaros raumenų atrofijos gydymas, esant 5q srities delecijoms
Maltese	Ċelluli proġenituri alloġeneiċi tal-motor newron imnissla minn ċelluli staminali embrijoniċi umani	Kura tal-atrofija muskolari spinali 5q

[1] At the time of designation

Language	Active ingredient	Indication
Polish	Allogeniczne komórki progenitorowe neuronu ruchowego uzyskane z ludzkich embrionalnych komórek macierzystych	Leczenie rdzeniowego zaniku mięśni 5q
Portuguese	Células alogénicas precursoras do neurónio motor derivadas de células estaminais de embrião humano	Tratamento da atrofia muscular espinal 5q
Romanian	Celule alogenice precursoare ale neuronilor motori provenite din celule stem embrionare umane	Tratamentul amiotrofiei spinale 5q
Slovak	Alogénne progenitorové bunky motorických neurónov derivované z ľudských embryonálnych kmeňových buniek	Liečba 5q spinálnej svalovej atrofie
Slovenian	Alogenične progenitorne celice motoričnih nevronov, pridobljene iz embrionalnih matičnih celic	Zdravljenje 5q spinalne mišične atrofije
Spanish	Células progenitoras de neuronas motoras alogénicas procedentes de células madre embrionarias humanas	Tratamiento de la atrofia muscular espinal 5q
Swedish	Allogena motorneuron-progenitorceller utvunna ur mänskliga embryonala stamceller	Behandling av 5q spinal muskelatrofi
Norwegian	Allogene motornevron progenitorceller avledet fra humane embryonale stamceller	Behandling av 5q spinal muskelatrofi
Icelandic	Ósamgena hreyfitaugunga stofnfrumur fengnar frá manna fósturvísa stofnfrumum	Meðferð við 5q mænuvöðvarýrnunar