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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous mesenchymal stromal cells on a decellularised tracheal scaffold from a cadaveric donor for the treatment of tracheal stenosis

On 29 August 2016, orphan designation (EU/3/16/1717) was granted by the European Commission to Videregen Ltd, United Kingdom, for autologous mesenchymal stromal cells on a decellularised tracheal scaffold from a cadaveric donor for the treatment of tracheal stenosis.

What is tracheal stenosis?

Tracheal stenosis is narrowing of the trachea, the windpipe that leads from the throat to the lungs. It can affect both children and adults. The disease can appear at birth or can occur later in life because of injury or certain diseases.

The severity of tracheal stenosis varies depending on the extent of the trachea affected, and symptoms include noisy breathing, shortness of breath and coughing.

Tracheal stenosis is a long-term debilitating and life-threatening condition because it can lead to lung infections and difficulty in swallowing and breathing.

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

At the time of designation, tracheal stenosis affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 103,000 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 medicines were authorised in the EU for the treatment of tracheal stenosis. Some patients underwent surgery to remove damaged areas of the trachea or medical procedures to try to dilate (widen) the trachea.

[*]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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How is this medicine expected to work?

This medicine is made of:

- a 'tracheal scaffold' from a dead donor, made only of the cartilaginous tube that supports the trachea with all other cells removed;
- cells called 'mesenchymal stromal cells' taken from the patient. These are cells able to develop into a variety of cell types, including osteoblasts (bone cells), chondrocytes (cartilage cells) and adipocytes (fat cells).

The mesenchymal stromal cells are first grown in the laboratory to increase their number and then seeded on the surface of the tracheal scaffold to form a working trachea. The damaged trachea is then removed from the patient and replaced with the seeded tracheal scaffold.

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, no clinical trials with the medicine in patients with tracheal stenosis had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for tracheal stenosis 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	Autologous mesenchymal stromal cells on a decellularised tracheal scaffold from a cadaveric donor	Treatment of tracheal stenosis
Bulgarian	Автоложни мезенхимални стромални клетки върху безклетъчна трахеална основа от трупен донор	Лечение на трахеална стеноза
Croatian	Autologne mezenhimalne stanice strome na decelulariziranom kosturu dušnika kadaveričnog donora	Liječenje stenoze dušnika
Czech	Autolognní mesenchymální stromální buňky na decelulalizován skeletonu trachey od kadaverózních dárců	Léčba tracheální stenózy
Danish	Autologe mesenkymale stromaceller på en matrix bestående af bindevæv fra trachea fra en afdød donor	Behandling af tracheostenosis
Dutch	Autologe mesenchymale stromale cellen op een gedecellulariseerd tracheal scaffold van een cadaver donor	Behandeling van tracheale stenosis
Estonian	Autoloogsed mesenhümaalsed stroomarakud kadaveerse doonori rakkudevabal trahheaskeletil	Trahhea stenoosi ravi
Finnish	Autologiset mesenkymaaliset stromaaliset solut desellularisoidulla henkitorvirungolla, joka on peräisin kuolleelta luovuttajalta	Henkitorven ahtauman hoito
French	Cellules stromales mésenchymateuses autologues sur matrice décellularisé (scaffold) de trachée issue d'un doneur cadaverique.	Traitement de la sténose trachéale
German	Autologe mesenchymale Stromazellen auf einem dezellularisierten trachealem Gerüst von einem Kadaverspender	Behandlung der Trachealstenose
Greek	Αυτόλογα μεσεγχυματικά στρωματικά κύτταρα σε αποκυτταρωμένο ικρίωμα τραχείας από πτωματικό δότη	Θεραπεία της στένωσης της τραχείας
Hungarian	Autológ mezenchymalis sztróma sejtek cadaver donortól származó decellularizált tracheális vázon	Légcsőszűkület kezelése
Italian	Cellule mésenchimali stromali autologhe su trachea decellularizzata (scaffold) da donatore cadaverico	Trattamento della stenosi tracheale
Latvian	Autologas mezenhīmas stromas šūnas uz trahejas karkasa, kas iegūts no miruša donora un atbrīvots no šūnām	Barības vada stenozes ārstēšana
Lithuanian	Autologinės mezenchiminės stromos ląstelės ant decelularizuoto trachėjos pagrindo iš mirusio donoro	Trachėjos stenozės gydymas
Maltese	Ċelluli stromali mesenkimali awtologuži fuq *scaffold* ta' trakea mingħajr ċelluli meħuda minn katavru	Kura ta' stenosi fit-trakea
Polish	Autologiczne mezenchymalne komórki zrębowe na pozbawionym komórek szkielecie tchawicy od zmarłego dawcy.	Leczenie zwężenia tchawicy
Portuguese	Células mesenquimais estromais autólogas num suporte traqueal descelularizado proveniente de cadáver de dador	Tratamento da estenose traqueal

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Celule mezenchimale stromale autologe pe matrice decelularizată (scaffold) de trahee provenită de la donator cadavru	Tratamentul stenozei traheale
Slovak	Autológne mezenchýmové bunky na decelularizovanej tracheálnej kostre od donora kadáveru	Liečba tracheálnej stenózy
Slovenian	Stromal cells on a decellularised tracheal scaffold from a cadaveric donor Avtologne meznehimske stromalne celice na decelulariziranem skeletu kadaverskega dajalca	Zdravljenje trahealne stenoze
Spanish	Celulas mesenquimales estromiales autologas sobre un suporte traqueal descelularizado de cadaver donante.	Tratamiento de la estenosis traqueal.
Swedish	Autologa mesenkymala stromaceller på en decellulerat trakea från kadaverdonator	Behandling av trakeal stenos
Norwegian	Autologe mesenkymale stromale celler på et decellularisert trakealt vevsskjelett fra død donor	Behandling av trakeal stenose
Icelandic	Samgena mesenkýmal strómalbarka palli frá látnum gjafa	Meðferð barka þrengsla