



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous urothelial and smooth muscle cells for the treatment of spinal cord injury

On 7 November 2008, orphan designation (EU/3/08/574) was granted by the European Commission to Choice Pharma Limited, United Kingdom, for autologous urothelial and smooth muscle cells, for the treatment of spinal cord injury.

The sponsorship was transferred to European Medical Advisory Services Limited, United Kingdom, in May 2011.

Please note that this product was withdrawn from the Community Register of designated orphan products in May 2012 on request of the sponsor.

What is spinal cord injury?

The spinal cord can be injured through accidents, such as damage to the back, or by internal causes such as tumours or bleeding within the spine putting pressure on the spinal cord. Injury to the spinal cord can damage the nerves that run through the cord and that branch out from it. This can stop the flow of nerve impulses between the brain and the body, resulting in the loss of feeling, paralysis and even death, depending upon the severity of the injury and where it is located. When the injury is to the lower part of the spinal cord, the bladder can be affected.

Spinal cord injury is life-threatening and chronically debilitating. The complications include paralysis of the legs with or without paralysis of the arms, breathing problems, blood clots in the veins and the lungs, and repeated infections of the lungs, the airways, the kidneys and the urinary tract (the structures that carry urine). Kidney and urinary tract infections are more common in patients whose bladder has been affected by the spinal cord injury.

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

At the time of designation, spinal cord injury affected between 2.2 to 4.2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of between 111,000 to 211,000 people, and is

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 502,282,000 (Eurostat 2008).



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 submission of the application for orphan drug designation, methylprednisolone was authorised for the treatment of spinal cord injury in some member states. If the bladder is affected by the spinal cord injury and becomes non-functional, a type of surgery called 'bladder augmentation' is sometimes used. This usually involves implanting tissue from the gut into the bladder to improve the function of it.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that the autologous urothelial and smooth muscle cells may be of significant benefit for the treatment of non-functional bladder in spinal cord injury. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Autologous urothelial and smooth muscle cells are obtained from the patient's (autologous) bladder. The cells are treated in a manufacturing process to create an artificial bladder around a synthetic scaffold. This is intended to substitute the non functional bladder and to avoid the need to use gastrointestinal segments during surgical augmentation of the bladder. It is thought that the cells will support and improve the function of the bladder.

What is the stage of development of this medicine?

The effects of autologous urothelial and smooth muscle cells were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with spinal cord injury had not been initiated.

Autologous urothelial and smooth muscle cells was not authorised anywhere worldwide for treatment of spinal cord injury or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 September 2008 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:

European Medical Advisory Services Limited
71 Knowl Piece
Wilbury Way
Hitchin
Hertfordshire SG4 0TY
United Kingdom
Telephone: +44 1462 424 400
Telefax: +44 1462 424 426
Contact: <http://www.emas-medical.com/contact.php>

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 languages¹, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Autologous urothelial and smooth muscle cells	Treatment of spinal cord injury
Bulgarian	Автоложни уротелиални и гладкомускулни клетки	Лечение на гръбначно-мозъчни травми
Czech	Autologní buňky urotelu a hladkého svalstva	Léčba míšního traumatu
Danish	Autologe urotelceller og glatte muskelceller	Behandling af rygmarvslæsion
Dutch	Autologe urotheelcellen en gladde spiercellen	Behandeling van ruggenmergletsel
Estonian	Autoloogsed uroepiteeli ja silelihase rakud	Seljaaju vigastuse ravi
Finnish	Autologisia uroteeli- ja sileälihassoluja	Selkäydinvamman hoito
French	Cellules urothéliales et cellules musculaires lisses autologues	Traitement des lésions de la moëlle épinière
German	Autologe Zellen des Urothels und der glatten Muskulatur	Behandlung der Rückenmarkverletzung
Greek	Αυτόλογα ουροθηλιακά και λεία μυϊκά κύτταρα	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Autológ urothelium- és simaizom-sejtek	Gerincvelő sérülés kezelése
Italian	Cellule uroteliali e cellule muscolari lisce autologhe	Trattamento delle lesioni del midollo spinale
Latvian	Autologas urīnceļu epitēlija un gludo muskuļu šūnas	Mugurkaula traumā ārstēšana
Lithuanian	Autologinės urotelio ir lygiųjų raumenų ląstelės	Nugaros smegenų sužalojimo gydymas
Maltese	Ċelluli uroteljali u ċelluli tal-muskoli lixxi awtologużi	Kura ta' korriment tan-nerv qawwi li jgħaddi minn ġos-sinsla
Polish	Autologiczne komórki nabłonka dróg moczowych i mięśni gładkich	Leczenie uszkodzenia rdzenia kręgowego
Portuguese	Células uroteliais e do músculo liso autólogas	Tratamento da lesão da medula espinal
Romanian	Celule musculare netede și uroteliale autologe	Tratamentul leziunilor măduvei spinării
Slovak	Autológne bunky urotelu a hladkého svala	Liečba poškodenia miechy
Slovenian	Avtologne celice urotelija in gladkih mišic	Zdravljenje poškodbe hrbtenjače
Spanish	Células autólogas uroteliales y musculares lisas	Tratamiento de las lesiones de la médula espinal
Swedish	Autologa uroteliala celler och glatta muskelceller	Behandling av ryggmärgsskada
Norwegian	Autologe urotelceller og celler i glatt muskulatur	Behandling av ryggmargsskade
Icelandic	Samgena þvagfæraþekju- og sléttvöðvafrumur	Meðferð mænuskaða vegna slyss

¹ At the time of designation