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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Autologous urothelial and smooth muscle cells for treatment of the spina bifida

On 17 March 2008, orphan designation (EU/3/08/537) was granted by the European Commission to Choice Pharma Limited, United Kingdom, for autologous urothelial and smooth muscle cells for treatment of spina bifida.

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Please note that this product was withdrawn from the Community Register of designated orphan products in May 2012 on request of the sponsor.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

The neural tube is the part of the embryo that gives origin to the nervous system. Its shape is similar to a tube and it is completely closed at a certain point of the embryonic development. Spina bifida is caused by the failure of the neural tube to close during the first month of embryonic development. This causes malfunction of some body functions controlled by the nervous system and include seizures, paralysis, limb deformity, bowel, bladder and sexual dysfunction. Spina bifida is chronically debilitating and life-threatening.

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

At the time of designation, spina bifida affected approximately 2.2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of 110,000 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 based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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What treatments are available?

There are no authorised medicinal products for the treatment of spina bifida in the Community. Treatment for the variety of effects of spina bifida may include surgery, medication, and physiotherapy. Surgery to close the newborn's spinal opening is generally performed within 24 hours after birth to minimize the risk of infection and to preserve existing function in the spinal cord.

Bladder function is normally controlled by some nerve roots from the spinal cord. In spina bifida, this control may be defective leading to a dysfunction of the urinary bladder (neurogenic bladder). Surgery such as bladder augmentation is sometimes used for the treatment of neurogenic bladder. A bladder augmentation is a surgical operation where tissue from gastrointestinal tract is used to improve the function of the bladder.

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 spina bifida. 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 spina bifida were initiated.

Autologous urothelial and smooth muscle cells was not authorised anywhere worldwide for treatment of spina bifida 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 6 February 2008 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:

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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Autologous urothelial and smooth muscle cells	Treatment of spina bifida
Bulgarian	Автоложни уротелиални и гладкомускулни клетки	Лечение на спина бифида
Czech	Autologní buňky urotelu a hladkého svalstva	Léčba spiny bifidy
Danish	Autologe urotelceller og glatte muskelceller	Behandling af spina bifida
Dutch	Autologe urotheelcellen en gladde spiercellen	Behandeling van spina bifida
Estonian	Autoloogsed uroepiteeli ja silelihase rakud	Spina bifida ravi
Finnish	Autologisia uroteeli- ja sileälihassoluja	Selkärankahalkion hoito
French	Cellules urothéliales et cellules musculaires lisses autologues	Traitement du spina bifida
German	Autologe Zellen des Urothels und der glatten Muskulatur	Behandlung von Spina bifida
Greek	Αυτόλογα ουροθηλιακά και λεία μυϊκά κύτταρα	Θεραπεία της δισχιδούς ράχης
Hungarian	Autológ urothelium- és simaizom-sejtek	Spina bifida kezelése
Italian	Cellule uroteliali e cellule muscolari lisce autologhe	Trattamento della spina bifida
Latvian	Autologas urīnceļu epitēlija un gludo muskuļu šūnas	Spina Bifida ārstēšana
Lithuanian	Autologinės urotelio ir lygiųjų raumenų ląstelės	Įskilo stuburo (spina bifida) gydymas
Maltese	Ċelluli uroteljali u ċelluli tal-muskoli lixxi awtologużi	Kura ta' l-ispina bifida
Polish	Autologiczne komórki nabłonka dróg moczowych i mięśni gładkich	Leczenie osób z rozszczepem kręgosłupa
Portuguese	Células uroteliais e do músculo liso autólogas	Tratamento de espinha bífida
Romanian	Celule musculare netede şi uroteliale autologe	Tratamentul Spinei Bifida
Slovak	Autológne bunky urotelu a hladkého svalu	Liečba spina bifida
Slovenian	Avtologne celice urotelija in gladkih mišic	Zdravljenje spine bifide
Spanish	Células autólogas uroteliales y musculares lisas	Tratamiento de la espina bífida
Swedish	Autologa uroteliala celler och glatta muskelceller	Behandling av spina bifida
Norwegian	Autologe urotelceller og celler i glatt muskulatur	Behandling av spina bifida
Icelandic	Samgena þvagfæraþekju- og sléttvöðvafrumur	Meðferð við klofnum hrygg

[1] At the time of designation