



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Ex-vivo expanded autologous human corneal epithelium containing stem cells for the treatment of limbal stem cell deficiency

On 17 July 2013, orphan designation (EU/3/13/1168) was granted by the European Commission to University Newcastle upon Tyne, United Kingdom, for ex-vivo expanded autologous human corneal epithelium containing stem cells for the treatment of limbal stem cell deficiency.

#### What is limbal stem cell deficiency?

Limbal stem cell deficiency is an eye condition in which the patient lacks cells called limbal stem cells, which are found at the edge of the cornea (the transparent layer in front of the eye) and which continuously renew and repair the cornea. The deficiency of limbal stem cells leads to clouding of the cornea and may result in impaired vision or blindness.

Limbal stem cell deficiency is a long-term disease that is debilitating to patients because of the impaired vision and discomfort it causes.

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

At the time of designation, limbal stem cell deficiency affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 people<sup>\*</sup>, 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, there were no satisfactory treatments authorised for treating the condition in the EU. Treatment being used included transplantation of limbal stem cells, obtained where possible from the patient's other eye.

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<sup>\*</sup>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).



## How is this medicine expected to work?

This product is made of cells that are taken from the surface of the patient's other eye and grown in the laboratory using a culture system containing cells derived from the human placenta. The cells form a layer called an epithelium, which is then implanted into the patient's damaged eye or eyes. The stem cells contained within the epithelium are then expected to help the cornea to regenerate, restoring the patient's vision.

## What is the stage of development of this medicine?

The effects of the medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicinal product in patients with limbal stem cell deficiency were ongoing.

At the time of submission, the medicinal product was not authorised anywhere in the EU for limbal stem cell deficiency 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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NE1 7RU  
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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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Ex-vivo expanded autologous human corneal epithelium containing stem cells	Treatment of limbal stem cell deficiency
Bulgarian	Ex-vivo развит автoloжен чoвешки рoгoвичен eпитeл, cъдържaщ cтвoлoви κeтκи	Лечeниe нa дeфицит нa лимбaлни cтвoлoви κeтκи
Czech	Autologní lidský rohovkový epitel expandovaný ex-vivo obsahující kmenové buňky	Léčba deficitu limbálních kmenových buněk
Croatian	Ex-vivo umnoženi autologni ljudski epitel rožnice koji sadrži matične stanice	Liječenje nedostatka limbalnih matičnih stanica
Danish	Ex vivo-ekspanderet autologt humant corneaepitel indeholdende stamceller	Behandling af limbal stamcellemangel
Dutch	Ex-vivo geëxpandeerd autoloog humaan corneaal epitheel dat stamcellen bevat	Behandeling van limbale stamceldeficiëntie
Estonian	Ex vivo kasvatatud autoloogne tüvirakke sisaldav inimese sarvkesta epiteel	Limbaalsete tüvirakkude puudulikkuse ravi
Finnish	Ex vivo kasvatettu autologinen, kantasoluja sisältävä, ihmisen sarveiskalvon epiteeli	Limbaalisen kantasolupuutoksen hoito
French	Épithélium cornéen humain autologue contenant des cellules souches et cultivé ex-vivo	Traitement du déficit en cellules souches limbiques
German	Ex vivo vermehrtes autologes menschliches Corneaepithel, das Stammzellen enthält	Behandlung der Limbusstammzellinsuffizienz
Greek	Ex-vivo καλλιεργημένο αυτόλογο ανθρώπινο επιθήλιο κερατοειδούς περιέχον βλαστοκύτταρα	Θεραπεία βλαστοκυτταρικής ανεπάρκειας του πρόσθιου θαλάμου
Hungarian	Ex.vivo szaporított őssejtek tartalmazó autológ humán szaruhártya hámszövet	Limbal őssejt hiány kezelése
Italian	Epitelio corneale umano autologo, espanso ex-vivo e contenente cellule staminali	Trattamento del deficit delle cellule staminali limbali
Latvian	Ex-vivo expanded autologous human corneal epithelium containing stem cells	Limbālo cilmes šūnu nepietiekamības ārstēšana
Lithuanian	Ex-vivo pagausintas autologinis žmogaus ragenos epitelis, turintis kamieninių ląstelių	Limbo kamieninių ląstelių stokos gydymas
Maltese	Epitelju awtologu uman tal-kornea mkabbar ex-vivo, li fih ċelluli staminali	Kura tan-nuqqas ta' ċelloli staminali limbali
Polish	Namnożony ex-vivo autologiczny ludzki nabłonek rogówki zawierający komórki macierzyste	Leczenie niedoboru komórek macierzystych rąbka rogówki

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Portuguese	Epitélio da córnea humana autólogo expandido ex-vivo contendo células estaminais	Tratamento da deficiência de células estaminais limbais
Romanian	Epiteliu autolog cornean uman expandat ex-vivo continand celule stem	Tratamentul deficientei de celule stem de la nivelul limbului
Slovak	Ex-vivo expandovaný autológny ľudský rohovkový epitel obsahujúci kmeňové bunky	Liečba deficiencie limbálnych kmeňových buniek
Slovenian	Ex vivo ekspandiran avtologen človeški roženični epitelij, ki vsebuje matične celice	Zdravljenje pomanjkanja limbalnih matičnih celic
Spanish	Epitelio corneal humano autólogo expandido ex-vivo que contiene células madre	Tratamiento de la deficiencia de células madre limbares
Swedish	Ex-vivo-expanderat autologt humant korneaepitel innehållande stamceller	Behandling av brist på limbala stamceller
Norwegian	Ex vivo-ekspandert autologt humant korneaepitel som inneholder stamceller	Behandling av mangel på limbale stamceller
Icelandic	Samgena manna glæru þekjuvefs stofnfrumur sem hafa vaxið ex vivo	Meðferð á limbal stofnfrumu skorti