

10 November 2014
EMA/COMP/557523/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cultured allogeneic corneal limbal stem cells for the treatment of limbal stem cell deficiency

On 15 October 2014, orphan designation (EU/3/14/1340) was granted by the European Commission to NHS National Services Scotland, trading as Scottish National Blood Transfusion Service, United Kingdom, for cultured allogeneic corneal limbal 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 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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, there were no satisfactory treatments authorised for treating the condition in the EU. Treatment being used included transplantation of limbal stem cells, which were obtained either from a donor or where possible from the patient's other eye.

*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 511,100,000 (Eurostat 2014).

How is this medicine expected to work?

This product is made of limbal stem cells that are taken from a donor's eye and grown on a membrane in the laboratory. The membrane is then implanted into the patient's damaged eye or eyes. The donor stem cells grown on the membrane 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 medicine have been evaluated in experimental models.

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

At the time of submission, the medicine 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 4 September 2014 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cultured allogeneic corneal limbal stem cells	Treatment of limbal stem cell deficiency
Bulgarian	култивирани алогенни роговични лимбални стволони клетки	Лечение на дефицит на лимбални стволони клетки
Croatian	Uzgojene alogene limbalne matične stanice rožnice	Liječenje nedostatka limbalnih matičnih stanica
Czech	Kultivované alogenní rohokové limbální kmenové buňky	Léčba deficitu limbálních kmenových buněk
Danish	Dyrkede allogene corneal limbal stamceller	Behandling af limbal stamcellemangel
Dutch	Gekweekte allogene corneaal limbale stamcellen	Behandeling van limbale stamceldeficiëntie
Estonian	Kultureeritud allogeensed sarvkesta limbaalsete tüvirakke	Limbaalsete tüvirakkude puudulikkuse ravi
Finnish	Viljeltyjä allogeenisia sarveiskalvon limbaalisia kantasoluja	Limbaalisen kantasolupuutoksen hoito
French	Cellules souches allogéniques cultivées du limbe cornéen	Traitement du déficit en cellules souches limbiques
German	Kultivierte allogene corneale limbale Stammzellen	Behandlung der Limbusstammzellinsuffizienz
Greek	Καλλιεργημένα αλλογενή βλαστοκύτταρα σκληροκερατοειδούς ζώνης	Θεραπεία βλαστοκυτταρικής ανεπάρκειας του πρόσθιου θαλάμου
Hungarian	Tenyészített allogén szaruhártya limbális őssejt	Limbal őssejt hiány kezelése
Italian	Cellule staminali corneali limbali allogeneiche coltivate	Trattamento del deficit delle cellule staminali limbali
Latvian	Kultivētas alogēnas radzenes limbālās cilmes šūnas	Limbālo cilmes šūnu nepietiekamības ārstēšana
Lithuanian	Dirbtinai išaugintos alogeninės ragenos limbo kamieninės ląstelės	Limbo kamieninių ląstelių stokos gydymas
Maltese	Ċelloli staminali limbali tal-kornea alloġeneici ikkolturati	Kura tan-nuqqas ta' ċelloli staminali limbali
Polish	Allogeniczne komórki macierzyste rąbka rogówki, namnożone	Leczenie niedoboru komórek macierzystych rąbka rogówki
Portuguese	Células estaminais alogénicas cornea-límbicas cultivadas	Tratamento da deficiência de células estaminais limbais
Romanian	Celule stem alogene de la nivelul limbului corneancultivate	Tratamentul deficientei de celule stem de la nivelul limbului
Slovak	Kultivované alogénne rohokové limbálne kmeňové bunky	Liečba deficiencie limbálnych kmeňových buniek
Slovenian	Vzgojene alogenske matične celice roženičnega roba	Zdravljenje pomanjkanja limbalnih matičnih celic

¹ At the time of designation

Language	Active ingredient	Indication
Spanish	Células madre limbares corneales alogénicas cultivadas	Tratamiento de la deficiencia de células madre limbares
Swedish	Odlade allogena kornea limbala stamceller	Behandling av brist på limbala stamceller
Norwegian	Dyrkede allogene kornea limbale stamceller	Behandling av mangel på limbale stamceller
Icelandic	Ræktaðar ósamgena glæru limbal stofnfrumur	Meðferð á limbal stofnfrumu skorti