

Limbal Stem Cell Plate Technology for Treatment of Intractable Limbal Stem Cell Deficiency

Definition

It is a disease caused by the lack of limbal stem cells capable of continuously regenerating corneal epithelium because extensive limbal damage occurs due to innate and acquired factors

Causes

Aniridia (innate factor) and acquired factors such as dry eye syndrome, atopic kerato-conjunctivitis, bullous keratopathy, trachoma, repeated eye surgery, diabetes, systemic chemotherapy (anticancer), radiation therapy, contact lenses, and ultraviolet exposure

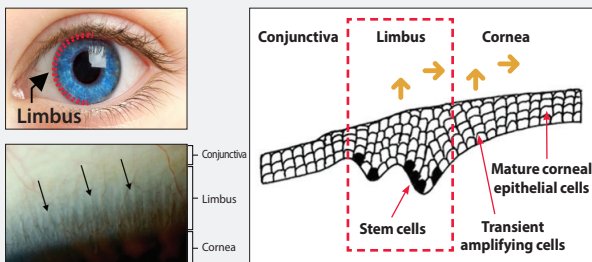
Symptoms

When limbal stem cells are lost due to extensive injury to the corneal limbus, conjunctival epithelium grows from the periphery of the cornea toward the cornea and is covered by conjunctival scar tissue. If these symptoms become chronic, corneal clouding occurs, resulting in decreased vision and blindness



Limbal Stem Cell Remedial Agent

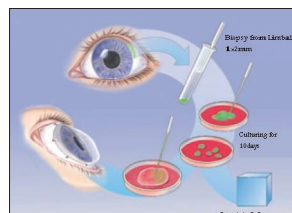
- Unipotent adult stem cells which are in the basal layer of the limbal epithelium, the boundary between the cornea and the bulbar conjunctiva.
- Cells with the ability to produce progenitor cells with differentiation potential into the corneal epithelium
- Progenitor cells migrate from the limbus to the corneal bottom layer and are involved in the regeneration of the corneal epithelium.



Anatomical location of the limbus. It is located between the cornea and the conjunctiva and stem cells are present at the bottom of Vogt Palisade (arrow).

Limbus-derived epithelial cell transplantation treatment

For limbal stem cell deficiency has a principle that the biopsy limbal tissue is cultured **in vitro** transplantation to make a cell plate that amplifies limbal stem cells, and then the stem cells are supplied by implanting them on the eyeball surface of the patient.



Differentiation Strategy

- It can be manufactured without a mouse feeder cell → **high safety**
- Autologous limbus-derived stem cell remedial agent → high safety and effectiveness → **high transplantation success rate**
- Development of human tissue (the amnion) and autologous limbuscultivation method → **economical and efficient**

Development Status

- Establishment and production of limbus-derived epithelial cell plate manufacturing process in GMP facility
- Application for MFDS phase II clinical trial → **In June 2021 it was IND approved.**
→ Technology transfer to CMO is currently in progress.

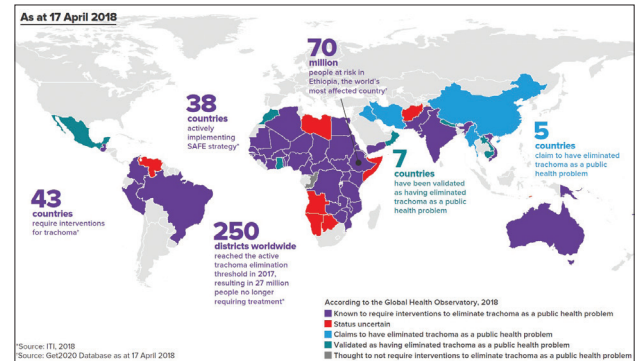
Domestic and International Competing Products

- As far as stem cell therapy has been approved, the only product that can be used for ophthalmologic diseases such corneal limbal deficiency is Holoclar of the Chiesi company in Italy, and the European Commission has conditionally allowed the introduction of the EU countries.
→ **100,000 Euros (approximately 130 million won) estimated**
- Excellent economic feasibility with differentiated manufacturing process/quality control → **High competitiveness**

Overseas Status - indication expansion

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- Trachoma is a representative disease that causes limbal stem cell deficiency and leads to blindness.
- About 8 million people worldwide lost their eyesight. As of now, 84 million people are already infected with the disease and need treatment.
- In addition, about 500 million people are at risk for the disease.

Domestic & International Applications and Registration Status

Classify	Patent name	Number	Date
Domestic Registration		10-1645901	Jun, 2016
International Application		PCT/KR2016/008003	Jul, 2016
US Application	Limbal stem cell cultivation method using amnion slide supporter	16/066,389	Jun, 2018
Europe Application		16881915.9	Jul, 2018
Japan Application		2018,553030	Jun, 2018
China Application		20160076831.4	Jun, 2018