Stem Cell Therapies in Ophthalmology: Promise and Perils

Dr. Muhammad Shaheer
Institute of Ophthalmology, Mayo Hospital Lahore.

Ophthalmol Pak. - Official Journal of College of Ophthalmology & Allied Vision Sciences

In recent years, ophthalmology as a specialty has seen phenomenal shift in its therapeutic landscape. Despite advancements in traditional surgical and pharmacological ocular interventions, degenerative and hereditary ocular disease treatment mainly remains palliative rather than curative. In this wake, stem cell therapies have emerged as a frontier not just to treat but also restore cellular morphology and physiology to potentially restore vision. Ophthalmology is probably best suited for this regenerative therapy as the eye is relatively more accessible, optically transparent and immune privileged thereby reducing the risk of rejection.

Cornea has been the first frontier historically with regards to stem cell therapy. Subsequent to ocular trauma, chemical burn etc, limbal stem cell deficiency is being successfully treated by grafting of harvested limbal stem cells from patient's healthy eye. Dental pulp of humans contain adult stem cells which like corneal stroma develop from the cranial neural crest. So theoretically they can also differentiate into keratocytes. Picard FNS and colleagues demonstrated such differentiation in mouse corneal stroma without affecting corneal transparency.[1]

Beyond cornea, Use of stem cells to treat degenerative and inherited retinal disease is also being widely investigated. In five clinical trials, anatomical and functional impact of stem cells on non neovascular Age related macular degeneration (NN-AMD) was observed.[2] In two trials, human umbilical cord derived stem cells were implanted via supra choroidal sub retinal delivery. Out of 35 subjects, 10 showed more than 10 letter gain while retinal detachment was noted in 6 patients. In other three trials, Human embryonic stem cells were implanted subretinally after pars plana vitrectomy. In these trials, only 4 subjects showed a gain of more than 15 letters. Likewise, in 4 clinical trials enrolling neovascular age related macular degeneration (N-AMD)[3], Human embryonic stem cells were injected after pars plana vitrectomy and majority of subjects showed stable vision with minimal side effects except for cystoid macular edema in one subject. Similarly human embryonic stem cells have been injected in subjects with stargart's disease. Surprisingly, the fellow eyes of these subject showed improvement in vision by at least 19 letters along with the treated eyes. Currently "OpRegen" (suspension of retinal pigment epithelial cells derived from human embryonic stem cells) is in phase 2 trials for its effectiveness in geographic atrophy.[4]

A 2024 systematic review and meta-analysis found that mesenchymal stem cell (MSC) therapy was associated with improvement in visual acuity in patients with optic neuropathy, though changes in retinal nerve fiber layer (RNFL) thickness were not consistent. This suggests that MSCs may have functional benefit, but structural regeneration remains more variable.[5] Preclinical and translational work is exploring MSC-derived extracellular vesicles (EVs) for optic neuritis. These EVs (exosomes) show anti-inflammatory and neuroprotective effects, and they can cross the blood–brain barrier.[6] In a meta-analysis carried out by Wo Du and associates, 19 studies showed that decreased IOP, increased Ganglion cell and Nerve fiber layer thickness in stem cell group as compared

How to Cite this Article:
Muhammad Shaheer, Stem Cell Therapies in Ophthalmology: Promise and Perils. Ophthalmol Pak. 2025;15(3):67-68.
DOI: https://doi.org/10.62276/OphthalmolPak.15.03.213

Correspondence: Muhammad Shaheer
Associate Professor, Institute of Ophthalmology, Mayo Hospital Lahore.
Email: mshaheer212@gmail.com
Received: 26-11-2025 **Accepted:** 26-11-2025

to controls. Such results may act as a supporting evidence supporting stem cell therapy as treatment modality for glaucoma.[7]

Diabetic retinopathy and inherited retinal disease are prevalent in Pakistan. Stem cell therapy may provide an alternate option where conventional treatment opportunities are limited. Likewise, availability of donor corneal tissue or access to transplantation infrastructure is limited. In this scenario, stem cell therapies can mitigate shortage and reduce dependence on donor grafts.[7] Though initially expensive, stem cell therapies might become cost-effective, especially if vision restoration reduces the social and economic burden of blindness. Despite conclusive and promising data, certain barriers are there such as biological risks i.e. tumorigenecity with pluripotent lines, technical barrier i.e. absence of certified culture facilities and strong ethical and regulatory concerns emphasizing the need for strict oversight to avoid "stem cell tourism" in Pakistan.

For Pakistan, stem cell therapy showcases a realistic frontier. Establishing regulatory infrastructure and investing in research capacity could heavily reduce the burden of blindness. Responsibly developed, stem cell therapy can shift ophthalmology from disease management toward true visual restoration.

REFERENCES:

1. Syed-Picard FN, Du Y, Lathrop KL, Mann MM, Funderburgh ML, Funderburgh JL. Dental pulp stem cells: a new cellular resource for corneal stromal regeneration. Stem cells translational medicine. 2015;4(3):276-85

2. Cao J, Murat C, An W, Yao X, Lee J, Santulli-Marotto S, et al. Human umbilical tissue-derived cells rescue retinal pigment epithelium dysfunction in retinal degeneration. Stem Cells. 2016;34:367–79.

3. Schwartz SD, Regillo CD, Lam BL, Eliott D, Rosenfeld PJ, Gregori NZ, et al. Human embryonic stem cell-derived retinal pigment epithelium in patients with age-related macular degeneration and Stargardt's macular dystrophy: follow-up of two open-label phase 1/2 studies. Lancet. 2015;385:509–16.

4. Trincao-Marques J, Ayton LN, Hickey DG, Marques-Neves C, Guymer RH, Edwards TL, et al. Gene and cell therapy for age-related macular degeneration: A review. Surv Ophthalmol. 2024;69(5):665-76.

5. Chaibakhsh, S., Azimi, F., Shoae-Hassani, A, . Evaluating the impact of mesenchymal stem cell therapy on visual acuity and retinal nerve fiber layer thickness in optic neuropathy patients: a comprehensive systematic review and meta-analysis. BMC Ophthalmol. 2024;24:316. https://doi.org/10.1186/s12886-024-03588-2

6. Aneesh, A., Liu, A., Moss, H.E. Emerging concepts in the treatment of optic neuritis: mesenchymal stem cell-derived extracellular vesicles. Stem Cell Res Ther. 2021;12:594 (2021). https://doi.org/10.1186/s13287-021-02645-7

7. Wu D, Liu Y, Zhang X, Zhang R, Wang S, Lu H, et al. Efficacy and safety of stem cells in the treatment of glaucoma: systematic review and meta-analysis based on animal experiments. Front Pharmacol. 2025;16:1587440.

8. Woo E, Henick D, Tahvildari M. Advancements in Cellular Therapeutics in Corneal Diseases. Cells.2025;14(23):1838. https://doi.org/10.3390/cells14231838.