



Allogeneic Müller Cells for the Treatment of Glaucoma

Müller cell therapy is a first in-class allogenic human embryonic stem cell derived therapy for advanced glaucoma.

Category
Cell Therapy

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Background

Glaucoma is a heterogeneous group of diseases characterised by progressive and irreversible sight loss. Primary Open-Angle Glaucoma (POAG) is the most common type of glaucoma and is associated with an increase in intraocular pressure (IOP). This increase in IOP leads to the death of Retinal Ganglion Cells (RGCs), which, along with their axons, form the optic nerve. Patients with POAG usually suffer from a slow, progressive increase in IOP and can remain asymptomatic for many years, allowing for significant damage to the optic nerve, and often for peripheral vision loss to occur before any medical treatment is sought (e.g., between 30 to 50% of RGCs may be lost before detection using standard visual field testing). Müller cells are the principal glial cells that provide support to all retinal cells, including RGCs, but this normal function is disrupted when the optic nerve gets damaged. Current treatments focus on lowering IOP, however, despite best efforts, many patients continue to develop visual impairment and eventually become blind.

Technology Overview

Allogeneic Müller cell therapy has been developed as an advanced cell therapy for glaucoma. Allogeneic Müller cells are derived from a GMP grade hESC line using a UCL proprietary GMP-compliant manufacturing process and have been shown to promote RGC axonal regeneration in vitro, express high levels of antioxidants and neurotrophins and significantly improve the visual function in NMDA-induced RGC-depleted glaucoma models (~50% improvement).

Allogeneic Müller Cell therapy has the potential to improve visual function in patients with advanced disease and also alter disease progression in patients at an earlier disease stage.

See Figure 1, Figure 2.

Stage of Development

- GMP Master Cell bank and ATMP product established from clinical grade hESC line and validated according to MHRA approved release criteria

- Ongoing product stability testing
- Completed non-GLP toxicology and biodistribution
- Phase 1 clinical trial designed
- Ready to start GLP toxicology study
- Phase 1 trial clinical trial design approved by UCL Clinical Trials Unit

Benefits

- Cryopreserved product injectable intravitreally
- Established proof of concept showing improvement of visual function in RGC-depleted glaucoma models
- GMP validated and transferable proprietary manufacturing process and know-how; scalable to thousands of doses from a single run
- Selected hESC line is available for future clinical and commercial development

Applications

The initial target market for the Müller cell therapy is patients with advanced glaucoma, where currently no treatments are available. Open-angle glaucoma OAG, which is the most common form of glaucoma, accounts for roughly 75–95% of all primary cases. It is estimated that 40% of POAG patients in the US and Europe will present with advanced disease and may be eligible for allogenic Müller cell therapy to prevent further disease progression and improve vision.

Opportunity

Currently available for licensing or co-development to complete IND enabling studies (GLP toxicology) and progress into Phase 1 clinical trial.

Patents (3)

- [US 17/773,103](#)
- [EP20801380](#)
- [JP2022525156](#)

Seeking

Licensing, commercial partner, development partner

IP Status

Patented,

Patent application submitted

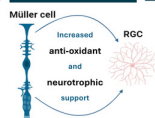
**Cryopreserved
product, frozen
supply**



Standard intravitreal injection



Trophic support released from cells



**Vision
enhancement/
maintenance**



The negative scotopic threshold response of the ERG (nSTR) at different light intensities indicates retinal ganglion cell function. Light intensities from -4.5 to -3 log cd second/m². nSTR calculated at the lowest point between 160-230ms. Plot shows least square mean \pm SEM.

