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Translating a stem cell therapy for age-related macular degeneration into the clinic: An update from the London Project to Cure Blindness

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1. Main Text

The London Project to Cure Blindness has developed a stem cell-based therapy for age-related macular degeneration (AMD), the leading cause of blindness in the developed world. Central to the project has been the generation of a functional retinal pigment epithelium (RPE) monolayer from human embryonic stem cells (HESCs)[1,2] under conditions suitable for clinical use[3]. This approach was translated into a Phase I/II clinical trial targeting patients with severe vision loss due to neovascular AMD.

2. Methods and results

In this trial, a hESC-derived RPE monolayer on a supportive synthetic scaffold was delivered subretinally in patients. Initial results demonstrated the safety and tolerability of the procedure, with signs of integration and functional benefit in patients[4]. More recently, a 5-year follow-up study confirmed long-term survival of the graft with no evidence of tumorigenicity, immune rejection, or significant adverse effects[5]. Complementary imaging and diagnostic modalities have also been used to assess structural outcomes and retinal remodelling[6]. These results mark a significant milestone in regenerative ophthalmology, supporting further clinical development of stem cell-derived therapies for retinal degenerative diseases.

3. References

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