Phase 1 clinical study of an embryonic stem cell-derived retinal pigment epithelium patch in age-related macular degeneration.

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Authors: Lyndon da Cruz, Kate Fynes, Odysseas Georgiadis, Julie Kerby, Yvonne H Luo, Ahmad Ahmado, Amanda Vernon, Julie T Daniels, Britta Nommiste, Shazeen M Hasan, Sakina B Gooljar, Amanda-Jayne F Carr, Anthony Vugler, Conor M Ramsden, Magda Bictash, Mike Fenster, Juliette Steer, Tricia Harbinson, Anna Wilbrey, Adnan Tufail, Gang Feng, Mark Whitlock, Anthony G Robson, Graham E Holder, Mandeep S Sagoo, Peter T Loudon, Paul Whiting, Peter J Coffey

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Public Summary:
Age-related macular degeneration (AMD) remains a major cause of blindness, with dysfunction and loss of retinal pigment epithelium (RPE) central to disease progression. We engineered an RPE patch comprising a fully differentiated, human embryonic stem cell (hESC)-derived RPE monolayer on a coated, synthetic basement membrane. We delivered the patch, using a purpose-designed microsurgical tool, into the subretinal space of one eye in each of two patients with severe exudative AMD. Primary endpoints were incidence and severity of adverse events and proportion of subjects with improved best-corrected visual acuity of 15 letters or more. We report successful delivery and survival of the RPE patch by biomicroscopy and optical coherence tomography, and a visual acuity gain of 29 and 21 letters in the two patients, respectively, over 12 months. Only local immunosuppression was used long-term. We also present the preclinical surgical, cell safety and tumorigenicity studies leading to trial approval. This work supports the feasibility and safety of hESC-RPE patch transplantation as a regenerative strategy for AMD.

Scientific Abstract:
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