Abstract

DC Discussion Group

Thursday, October 5, 2017
IBBR

Kapil Bharti
National Eye Institute, National Institutes of Health

Developing an Autologous Cell Therapy for Macular Degeneration

Induced pluripotent stem (iPS) cells are a promising source of personalized therapy. These cells can provide immune-compatible autologous replacement tissue for the treatment of potentially all degenerative diseases. We preparing for the first phase I clinical trial using iPS cell derived ocular tissue to treat age-related macular degeneration (AMD), one of the leading blinding diseases in the US. AMD is caused by the progressive degeneration of retinal pigment epithelium (RPE), a monolayer tissue that maintains vision by maintaining photoreceptor function and survival. Combining developmental biology with tissue engineering we have developed clinical-grade iPS cell derived RPE patch on a biodegradable scaffold. Clinical-grade iPS cells derived from AMD patients are characterized for purity, identify, karyotyping, and absence of potentially oncogenic mutations. And, clinical-grade RPE cells are characterized for purity (flow cytometry for RPE markers), epithelial shape (computational based image analysis), electrical and mechanical intactness of the monolayer (trans-epithelial resistance), polarized secretion of cytokines (higher basal/apical VEGF secretion), and ability to phagocytose outer segments (flow based phagocytosis of photoreceptor outer segments). The clinical-grade iPSC-RPE patch has been qualified using all of these assays. Currently, we are testing the safety and the efficacy of this replacement patch in animal models as part of a Phase I Investigational New Drug (IND)-application. Approval of this IND application will lead to transplantation of autologous iPS cell derived RPE patch in patients with the advanced stage of AMD.