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In vitro disease model of dry AMD

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Age-related macular degeneration (AMD) is the leading cause of blindness in people over age 55 in the U.S. and the developed world. AMD is a multifactorial disease and there is no adequate animal model that recapitulates key characteristics of dry AMD. The lack of a robust model for AMD is a major impediment for mechanistic studies and drug development. Consequently, effective treatments for AMD are not available, although vitamin supplementation is recommended and is modestly beneficial for a small population of patients. Therefore, the need for novel therapeutic and preventive strategies is pressing. Recent generation of RPE cells from embryonic stem cells (ESCs) and induced pluripotent stem cells (iPSCs), offers new promise for cell replacement therapy and disease modeling in AMD. Previously, we showed that the induced pluripotent stem cell-derived retinal pigment epithelium (RPE), (iPSC-RPE) are phenotypically and functionally identical to the native RPE, offering promise for cell replacement therapy in AMD. We recently generated an iPSC-derived disease model for dry AMD. AMD Patients' skin cell were collected and cultured under an IRB protocol and induced to pluripotency using the Yamanaka vectors, prior to differentiation into RPE. Striking disease phenotypes and impaired functions were identified in AMD iPSC-RPE compared to normal iPSC-RPE. The mechanisms underlying the disease phenotypes have been investigated. Our research elucidates whether iPSC-RPE from skin of AMD patients are a reliable and safe source for autologous cell-based therapy in AMD, and proposes important new information for development of novel drugs for treatment of dry AMD.

Biography

Nady Golestaneh has earned her PhD in 2000, from University of Paris VI, Pierre et Marie Curie, Paris France, her Master Degree from University of Paris VI, Pierre et Marie Curie, Paris France and her Bachelor Degree from University of Paris VII, Jussieu, Paris France. She performed her Postdoctoral training at NEI/NIH, Johns Hopkins University and Georgetown University. She is Director of Research at the Department of Ophthalmology at Georgetown University Medical Center and Assistant Professor at Departments of Ophthalmology, Neurology, Biochemistry and Molecular & Cellular Biology at Georgetown University School of Medicine. She has published over 29 peer reviewed papers and has received several national and international awards. She serves as Editorial Board Member of *Human Genetics & Embryology*, *International Journal of BioEngineering and Technology*, and *CellR4*.

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