Stem cell-derived organoids for testing gene delivery to retinal and photoreceptor cells

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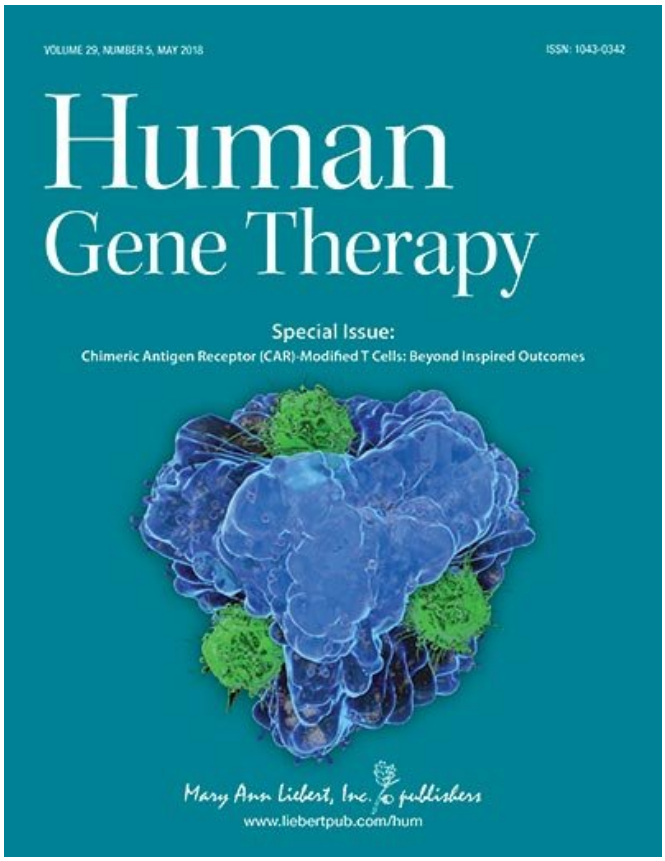

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A new study that compared six of the most promising adeno-associated viral (AAV) gene therapy vectors in human retinal organoid models showed clear distinctions in the efficiency of gene transfer to both retinal pigment epithelial (RPE) and photoreceptor cells. The results of this study can help guide future selection and design of viral vectors for therapeutic gene delivery and gene editing and are reported in an article published in *Human Gene Therapy*.

Robin Ali, University College London (UCL) and President of the European Society of Gene and Cell Therapy (ESGCT) and colleagues from UCL and University of Edinburgh, U.K. coauthored the article entitled "Assessment of AAV Vector Tropisms for Mouse and Human Pluripotent Stem Cell-Derived RPE and Photoreceptor Cells." The research-ers demonstrated the feasibility of using human pluripotent stem cell-derived retinal or-ganoids as an in vitro test system for the development of gene therapy vectors.

"One of the most widespread uses of human pluripotent stem cells in translational re-search has been to create well-differentiated living human tissues and organs 'in a dish' so that they may be used to test the relative efficacy of various drugs prior to their use in patients," says Editor-in-Chief Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School, Worcester, MA. "These studies by Dr. Ali have ex-tended that concept to the hottest application in gene therapy today, the use of AAV vec-tors in the eye."

More information: Anai Gonzalez-Cordero et al, Assessment of AAV Vector Tropisms for Mouse and Human Pluripotent Stem Cell–Derived RPE and Photoreceptor Cells, *Human Gene Therapy* (2018). DOI: 10.1089/hum.2018.027

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