RETINAL ORGANOID MODEL SYSTEMS

View U.S. Patent Application Publication No. US-2023-0151327 in PDF format.

WARF: P210058US02

Inventors: David Gamm, Steven Mayerl

The Invention

UW-Madison researchers have developed a retinal organoid model system for evaluating therapies that target genetic mutations. The inventors have discovered that genetic modifications that compromise the expression of the gene IMPG2 result in the loss of a readily visible phenotype (hair-like photoreceptor structures) when engineered into stem cells that are used to form retinal organoids using the inventors' previously-developed retinal organoid model system; therapies that correct the incorporated genetic modifications are readily identifiable as rescuing the phenotype. The present assay can be used with a variety of modifications that disrupt gene expression (e.g., missense, nonsense, frameshift, cryptic splice variants, coding/non-coding, etc.). The cells of the treated organoids can be sequenced to evaluate whether a therapy creates off-target effects, and thereby provides a generic readout of efficacy and certain measures of safety for gene, genome, and/or RNA-based therapeutics.

Additional Information

For More Information About the Inventors

- David Gamm

Tech Fields

- Drug Delivery : Other drug delivery technologies

For current licensing status, please contact Andy DeTienne at adetienne@warf.org or 608-960-9857