3rd Retinal Cell And Gene Therapy Innovation Summit

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Retinal repair: Bringing stem cells into focus Retinal Blindness, Gene Therapy, CRISPR, and Artificial Intelligence

Retinal Research Update - 4/18/2020Retinal regeneration and photoreceptor reprogramming My Retina Tracker Registry Open Access Genetic Testing *Retinal Progenitor Cells for Treatment of Retinitis Pigmentosa - Henry Klassen ReNeuron: Using hRPCs*

to treat inherited retinal degenerative diseases Can stem cells restore vision in retinal degeneration? Gene Therapy for Inherited Retinal Diseases Clinical Embryology of The Eye - Part 1 (Basics) *Ophthotech: Transformative Gene Therapy and Novel Therapeutics in Retina*

GenSight: Disruptive Gene Therapy in Retinal and Neurodegenerative Disease

Retinitis Pigmentosa Eye Exercise to Improve Eyesight *Will Human Retinas Be Able to Regenerate?* White Board: Retinitis Pigmentosa (RP) *Gene Therapy Explained* Dr. Peter Campochiaro | Retina Specialist

Using CRISPR to reverse retinitis pigmentosa and restore visual function | QPTjCyte Developing Cell-

based RP Treatment at Ophthalmology Innovation Summit @ AAO jCyte #OpenUpStemCells: Prof. Christelle Monville on stem cells and retina repair **Bionic Eyes Give Second Sight to the Blind** *Scientists regenerate retinal cells in mice Bart Leroy, EURETINA 2019 - Updates in retinal gene therapy* Nightstar Taking Many Shots at Retinal Disease Professor Robin Ali on gene and cell therapies to prevent blindness due to retinal disease **3. Retinal Cell Fate Determination**

ReNeuron Group - Gene \u0026 Cell Therapy Spotlight at OIS @ AAO 2019Retinal Gene Therapy 2018 at Ophthalmology Innovation Summit Retina - OIS **Reversing Blindness with New FDA Approved**

Retinal Gene Therapy 3rd Retinal Cell And Gene
Gene therapy approaches to treating inherited retinal diseases are of special interest given the accessibility of the eye, its immune-privileged status and the successful clinical trials of RPE65 ...

Restoration of retinal and visual function following gene ...

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and gene ...

3rd Retinal Cell And Gene Therapy Innovation Summit

Gene therapy approaches to treating inherited retinal diseases are of special interest given the accessibility of the eye, its immune-privileged status and the successful clinical trials of RPE65 ...

Study reveals restoration of retinal and visual function ...

A retinal dose of AAV could contain 300-500 billion capsids. Not all capsids will make it into the nucleus of the retinal cell — where they need to be to work — and some capsids don't have cargo. That's why so

many capsids need to be in the bleb for enough therapeutic gene to get into the retinal cells.

A Gene Therapy Primer for People with Inherited Retinal ...

Second, retinal cells do not proliferate after birth. This is important as a single injection could potentially offer life-long expression of the therapeutic protein. Third, a number of animal models are available for inherited retinal diseases, which is instrumental for safe and efficient drug development.

Retinal Gene Therapy - svarlifescience.com

We identified all major retinal cell types in each

species, including a subset of zebrafish rod-restricted progenitor cells that expressed nr2e3 and neurod1 (Fig. 2D and fig. S2, F and G). Rod...

Gene regulatory networks controlling vertebrate retinal ...

Retinal cell replacement would be valuable for regenerating functional retinas, and therefore it is being examined as a next-generation treatment for retinal degeneration. With advances in stem cell biology, considerable progress has been made in recent years on generation of retinal cells.

Retina Degeneration - an overview | ScienceDirect

Topics

Summary. Biallelic mutations in the RPE65 gene are associated with inherited retinal degenerations/dystrophies (IRD) and disrupt the visual cycle, leading to loss of vision. A new adenoviral vector-based gene therapy surgically delivered to retinal cells provides normal human RPE65 protein that can restore the visual cycle and some vision.

Gene Therapy for Retinal Degeneration: Cell

Furthermore, retroviral gene transfer of Otx2 steers retinal progenitor cells toward becoming photoreceptors. Thus, Otx2 is a key regulatory gene

for the cell fate determination of retinal photoreceptor cells. Our results reveal the key molecular steps required for photoreceptor cell-fate determination and pinealocyte development.

Otx2 homeobox gene controls retinal photoreceptor cell ...

The summit features presentations by leading retinal disease experts on potential gene and stem-cell therapies and how best to deliver them to patients. “The purpose of the summit is to create visibility for the many projects based on gene or cell therapy approaches that are in or entering the clinic,” Brian Mansfield , PhD, executive vice president, interim

chief scientific officer, said ...

Foundation Fighting Blindness to Host Annual Retinal Cell ...

Most projects involve retinal gene therapy, which is not surprising considering the number of advantages the eye offers. As a consequence of this rise in interest, we have added information on our website about the background of retinal gene therapy and how our products and services can help you develop safe and reliable gene therapy products.

All eyes on Retinal Gene Therapy

Inherited retinal dystrophies are a group of eye

diseases caused by gene mutations which result in the gradual degeneration of the light sensitive cells (photoreceptor cells) on the back of the eye (the retina). The RPE65 gene provides instructions for making a protein that is essential for normal vision. RPE65-mediated inherited retinal dystrophies are rare and serious.

NICE recommends novel gene therapy treatment for rare ...

Gene expression of Nestin, paired box protein 6 (PAX6), Thy1 and brain-specific homeobox/POU domain protein 3 (Brn-3) in retinal progenitor cells was detected by reverse transcription-quantitative

polymerase chain reaction.

Retinal ganglion cell-conditioned medium and surrounding ...

The gene, which is called RPE65, is injected into the eye, under the retina, in an operating room procedure performed by Dr. Maguire. The gene enters retinal cells because it is packaged into a safe virus called adeno-associated virus (AAV).

Gene Therapy for Macular Degeneration & Other Eye Diseases ...

More information: Subrata Batabyal et al, Sensitization of ON-bipolar cells with ambient light

activatable multi-characteristic opsin rescues vision in mice, Gene Therapy (2020). DOI: 10.1038 ...

Scientists use gene therapy and a novel light-sensing ...

This is achieved by using a harmless virus known as adeno-associated virus, or AAV, to carry normal genes into the retinal cells. In 2009, the team commenced the development of AAV gene therapies for treatment of choroideremia and X-linked retinitis pigmentosa (RP), incurable genetic diseases that cause blindness in men.

Creation and spinout of Nightstar, a retinal gene

therapy ...

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New method to treat blindness using retinal cell production

UK-based biotech ReNeuron has announced encouraging results from an early stage trial of its cell therapy for the rare blindness-causing disease, retinitis pigmentosa.

Cell therapy produces encouraging first results in eye

trial

The company currently has three ongoing clinical programs for IRD products, with a fourth program expected to enter clinical development along with its pre-clinical IRD pipeline. Janssen will also enter a research collaboration with MeiraGTx covering the former's pre-clinical IRD program pipeline.

Janssen partners for gene therapy development

Please note that the seventh annual Retinal Cell and Gene Therapy Innovation Summit previously scheduled for Friday, May 1st, 2020 in Baltimore, Maryland has been cancelled. Close. National Fall Virtual VisionWalk . October 24, 2020.

Inherited retinal degenerations are genetically heterogeneous conditions affecting roughly 1:3000 people and are characterized by the loss of photoreceptors. Progressive retinal degenerative disease is the leading cause of vision loss in industrialized countries, and is the result of a wide range of mutations, mostly in rod-specific transcripts. Over 140 disease-causing genes have been identified to date. As the genetic mechanisms underlying inherited forms of retinal degeneration are identified, gene therapy is becoming a promising approach for

the treatment of many inherited blinding diseases. Indeed, the recent success of three clinical trials using adeno-associated virus (AAV) to deliver a normal copy of the RPE65 gene to the retinas of Leber congenital amaurosis (LCA) patients illustrates the potential of gene therapy in the retina. AAV has been shown safe and effective especially in a younger cohort of patients. Some important obstacles remain, however, for AAV-mediated gene therapy to become widely applicable across the range of existing retinal degenerative diseases. It will be essential to carefully evaluate the method used to deliver therapeutic genetic material to the retina, as this will determine the success of the treatment. The serotype of vector

used, the promoter chosen to drive expression and the method of injection are important components of the gene delivery system. A wide variety of AAV serotypes exist with different tropisms for cell populations in the retina, potentially allowing treatments to be targeted to specific cell types. The retinal cell types AAV can infect differs, however, depending on whether the vector is delivered into the vitreous cavity or the subretinal space. Subretinal injections, which were used in the LCA trials, result in the creation of a retinal detachment and localized injury to the retina while delivering high concentrations of transgene to only a limited area. An intravitreal approach has the potential to transduce

panretinally and is less invasive, and therefore preferable, but naturally occurring serotypes of AAV transduce photoreceptors poorly from the vitreous, as a result of structural barriers that exist on the inner surface of the retina. Recent advances in the understanding of AAV and the production of viral vectors have shown the flexibility of this virus, indicating that its function can be altered and tailored to the requirements of retinal gene therapy. A directed evolution approach has been used to select, out of a highly diverse library of AAV capsid variants, a novel variant with improved tropism for Müller glia. And in a parallel approach, residues on the capsid surface have been mutated to avoid ubiquitination

and altering the nuclear trafficking of the virus. This dissertation examines the use of engineered viral vectors for gene therapy in the retina. The creation of a novel variant of AAV, called 7m8, which is characterized by increased transduction of photoreceptors from the vitreous, is described below. 7m8 was derived from an AAV2 peptide insertion library and contains a 7mer motif. Injected intravitreally, 7m8 transduces cells throughout the retina, including photoreceptors in the outer retina, significantly more efficiently compared to the parental serotype. Expression was restricted to photoreceptors using a rhodopsin promoter. This virus, as well as the previously described Müller-specific variant ShH10,

was used to deliver a wild-type copy of the retinoschisin gene to mice lacking this protein. Retinoschisin is secreted from photoreceptors, and retinas deficient in this protein are severely structurally impaired. Subretinal injections, which are damaging in nature, are therefore suboptimal because they are likely to cause additional injury. We show that 7m8 is able to efficiently target photoreceptors via intravitreal injection in this mouse model, leading to high levels of retinoschisin protein production, as well as structural and functional rescue. This rescue is longer lasting than that seen using ShH10, indicating the importance of targeting photoreceptors in this disease model. AAV9 has been

shown to transduce the murine retina when injected intravenously through the tail vein. We used two surface tyrosine-to-phenylalanine mutations to improve the retinal expression of AAV9, and demonstrated that these mutations lead to higher infectivity of all retinal layers, most dramatically in photoreceptors and the inner nuclear layer, but also including the retinal pigment epithelium and ganglion cells. This novel vector was then used to explore the bifunctionality of the Nxnl1 gene, which encodes two isoforms of the rod-derived cone viability factor (RdCVF). The short form of RdCVF is secreted and has been shown to support cone survival, while the long isoform is retained intracellularly and has been

implicated in redox signaling. AAV92YF and 7m8 were used to express the two isoforms of RdCVF in the rd10 mouse model of retinitis pigmentosa. RdCVF rescued cone survival when injected intravenously or intravitreally, but had little effect on rod survival. Early expression of RdCVFL in dark-reared rd10 mice delayed rod, and subsequently cone death.

Glaucoma is a neurodegenerative disease that can lead to a complete loss of vision due to retinal ganglion cell (RGC) death. Therapies that have the capacity to protect and rescue stressed RGCs remain a critical unmet need in glaucoma management. Neurotrophic factor (NF) gene therapy is a promising

therapeutic approach that can address this current clinical deficiency by providing damaged RGCs with extrinsic neurotrophic support as a means of protection and repair. Moreover, a non-viral approach to the delivery of NF-encoding plasmid DNA (pDNA) confers many advantages over a viral approach for its improved immunogenicity and mutagenesis risks, patient compliance and large-scale manufacturing cost and feasibility. In this research, the main objective was to address the challenges facing non-viral NF gene therapy field for the retina, through development of three in vitro model systems that aim to facilitate the preclinical screening and identification of promising NF gene delivery systems. The first

model system developed was a versatile co-culture model that simulates cellular interactions between "healthy" and "stressed" cells in the retina. Furthermore, through incorporation of techniques including enzyme-linked immunosorbent assay (ELISA), immunofluorescent imaging, and neurite tracing into the co-culture setup, the model system enables a systematic evaluation of the therapeutic potential of gene delivery systems through assessment of bioavailability and bioactivity of therapeutic proteins produced from transfected cells. The second model system was a new potential RGC cell line, termed XFC series of cells, that express key RGC characteristics and suitable for the evaluation of

RGC-aimed gene therapies. Derived from multipotent retinal stem cells (RSCs), XFC cells express multiple RGC markers including Map-2, Rbpms, and Tubb3, and exhibit RGC-like neurite extension capacity in response to brain-derived neurotrophic factor (BDNF), ciliary neurotrophic factor (CNTF), and rho-kinase inhibitor (RKI) Y-27632 activation. The feasibility of the cell model was further validated in the described co-culture setup as XFC cells were able to validate the bioactivity of the BDNF proteins released by transfected cells. The third model system developed was a stem cell-derived 3D "mini-retina" culture model (termed MiEye series of retinal neurospheres) that contains multiple retinal cell types and enables in

vivo-like gene delivery assessment. Derived from differentiating multipotent RSCs in 3D culture, MiEye retinal neurospheres with different retinal biomarker expressions can be generated using different protocols. Moreover, by harnessing the tissue-like arrangement of retinal cells in MiEye retinal neurospheres, it enables the assessment of infiltration and transfection capacity of gene delivery systems in tissue-like structure, towards the establishment of a more representative in vitro-in vivo correlation and prediction of in vivo gene delivery feasibility. Concurrent to model system development, aspects that focus on the development of non-viral gene delivery systems for the retina were also explored.

The first aspect involved the optimization of gemini surfactant (GS) lipid nanoparticle systems (GL-NPs) physicochemical properties by evaluating the roles of minicircle plasmid (MC), sonication processing, and total NP component concentration (TNPC). Through dynamic light scattering and fluorescent correlation spectroscopy physicochemical characterizations, it was found that the size, particle size distribution, and zeta potential could be effectively optimized through sonication processing and TNPC. Moreover, the number of pDNA per particle homogeneity can be improved by formulating GL-NPs with MCs. The second aspect involved an investigation on the application of carbon nanotubes (CNTs) as a gene

delivery vehicle to retinal cell types. More specifically, GS-functionalized SWNT gene delivery system (ƒ-ptSWNT) was developed and demonstrated the ability to deliver pDNA to a retinal astrocyte cell line. The results demonstrate the feasibility of utilizing ƒ-ptSWNT to deliver pDNA to retinal cells and serves as a starting point for future ƒ-ptSWNT retinal gene delivery system development. The development of the three in vitro model systems in this thesis collectively aims to facilitate the preclinical screening and development of non-viral NF gene therapies in a synergistic manner, covering key areas of assessments that are critical to in vivo therapeutic success. Furthermore, concurrent developments in

non-viral gene delivery systems through GL-NP optimization and CNT exploration also advance the knowledge towards the development of better non-viral gene delivery systems.

A discussion of all the key issues in the use of human pluripotent stem cells for treating degenerative diseases or for replacing tissues lost from trauma. On the practical side, the topics range from the problems of deriving human embryonic stem cells and driving their differentiation along specific lineages, regulating their development into mature cells, and bringing stem cell therapy to clinical trials. Regulatory issues are addressed in discussions of the ethical debate

surrounding the derivation of human embryonic stem cells and the current policies governing their use in the United States and abroad, including the rules and conditions regulating federal funding and questions of intellectual property.

Abstract Development and Assessment of Gene Therapies for Inherited Blinding Diseases By Kathleen Durgin Kolstad Doctor of Philosophy in Molecular and Cell Biology University of California, Berkeley Professor John Flannery, Committee Chair There are two therapeutic approaches for inherited retinal

disease addressed in this dissertation: we sought to slow retinal degeneration and reverse visual loss after complete photoreceptor apoptosis. In the first approach, by viral gene transfer to the support cells of the retina, Müller glia (RMCs), we achieved sustained secretion of human glial derived neurotrophic factor (hGDNF) (Chapter 3). We hypothesized that hGDNF production by retinal glia will enhance the protective affects of RMCs in the diseased retina and help slow photoreceptor degeneration. Furthermore, this method avoids extra photoreceptor stress caused by direct hGDNF gene transfer to cells that are already stressed. We were able to optimize gene transfer to RMCs and observe

the beginnings of functional rescue in an animal model of autosomal dominant retinitis pigmentosa with this technique. One major advantage of this therapeutic approach is that it is applicable to multiple retinal disease genotypes. The second approach to ocular gene therapy presented in this dissertation was to re-introduce photosensitivity to the retina after complete photoreceptor degeneration (Chapter 4). To this end, we employed the engineered light activated glutamate receptor (LiGluR) to confer light sensitivity on retinal ganglion cells (RGCs) in the diseased retina. We first showed LiGluR mediated RGC photo-activation in in vitro retinal tissue preparation. We then characterized in vivo cell

population responses (visually evoked potentials, VEPs) in V1 when retinal input was limited to LiGluR induced activity in the retina. VEPs driven by LiGluR are approximately 50% of the amplitude of full field light flash driven responses in the wild type animal. LiGluR driven cortical responses in blind animals suggest that it is a promising therapy for restoring visual function and processing in the late stages of retinal degeneration. In the third part of this dissertation (Chapter 2a and 2b), the goal was to develop and assess methods of making ocular gene therapies safer and more efficacious. Current gene therapies for retinal degenerative diseases rely on subretinal delivery of viral vectors carrying

therapeutic DNA. However, this method of delivery limits the viral transduction profile to the region of injection and seriously compromises the retina during detachment. We have identified natural barriers to viral vector delivery to the outer retina from the vitreous. Furthermore, we have developed artificial methods and characterized disease states that allow these barriers to be overcome. The understanding of and the ability to manipulate barriers to vitreal delivery of viral vectors will help avoid the limitations, risks, and damage associated with subretinal injections.

Regenerative medicine – stem cell and gene-based

therapy – offers a new approach for restoring function of damaged organs and tissues. This is the first book to cover the major new aspects and field of regenerative medicine. This title is therefore a timely addition to the literature. It brings together the major approaches to regenerative medicine in one text, which ensures that techniques learnt in one discipline are disseminated across other areas of medicine.

Virtually any disease that results from malfunctioning, damaged, or failing tissues may be potentially cured through regenerative medicine therapies, by either regenerating the damaged tissues in vivo, or by growing the tissues and organs in vitro and

implanting them into the patient. Principles of Regenerative Medicine discusses the latest advances in technology and medicine for replacing tissues and organs damaged by disease and of developing therapies for previously untreatable conditions, such as diabetes, heart disease, liver disease, and renal failure. Key for all researchers and instituions in Stem Cell Biology, Bioengineering, and Developmental Biology The first of its kind to offer an advanced understanding of the latest technologies in regenerative medicine New discoveries from leading researchers on restoration of diseased tissues and organs

This book will contain the proceedings of the XIV International Symposium on Retinal Degeneration (RD2010), held July 13-17, 2010, in Mont-Tremblant, Quebec, Canada. The volume will present representative state-of-the-art research in almost all areas of retinal degenerations, ranging from cytopathologic, physiologic, diagnostic and clinical aspects; animal models; mechanisms of cell death; candidate genes, cloning, mapping and other aspects of molecular genetics; and developing potential therapeutic measures such as gene therapy and neuroprotective agents for potential pharmaceutical therapy.

This Atlas of Inherited Retinal Disorders provides a thorough overview of various inherited retinal dystrophies with emphasis on phenotype characteristics and how they relate to the most frequently encountered genes. It also meets the previously unmet needs of PhD students who will benefit from seeing the phenotypes of genes they work on and study. Further, because genetic-testing costs are quite high and spiraling higher, this Atlas will help geneticists familiarize themselves with the candidate gene approach to test patients' genomes, enabling more cost-efficient testing. This invaluable atlas is organized into eight sections starting with an introduction to the basic knowledge on retinal

imaging, followed by diseases listed according to inheritance pattern and disorders with extraocular manifestations grouped by defining features. This structure will be intuitive to clinicians and students studying inherited retinal disorders.

Pituitary Adenylate Cyclase-Activating Polypeptide is the first volume to be written on the neuropeptide PACAP. It covers all domains of PACAP from molecular and cellular aspects to physiological activities and promises for new therapeutic strategies. Pituitary Adenylate Cyclase-Activating Polypeptide is the twentieth volume published in the Endocrine Updates book series under the Series Editorship of Shlomo

Melmed, MD.