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Cell And Gene Therapy

# **3rd Retinal Cell And Gene Therapy Innovation Summit**

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Retinal repair: Bringing stem cells into focus Retinal Blindness, Gene

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Therapy, CRISPR, and Artificial  
Intelligence

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Retinal Research Update - 4/18/2020

~~Retinal regeneration and~~

~~photoreceptor reprogramming My~~

~~Retina Tracker Registry Open Access~~

~~Genetic Testing *Retinal Progenitor*~~

~~*Cells for Treatment of Retinitis*~~

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*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*

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*Gene Therapy and Novel Therapeutics  
in Retina*

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GenSight: Disruptive Gene Therapy in  
Retinal and Neurodegenerative  
Disease

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Retinitis Pigmentosa Eye Exercise to  
Improve Eyesight *Will Human Retinas  
Be Able to Regenerate?* ~~White Board:~~

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~~Innovation Summit~~ *Retinitis Pigmentosa (RP) Gene  
Therapy Explained* Dr. Peter  
Campochiaro | Retina Specialist

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Using CRISPR to reverse retinitis  
pigmentosa and restore visual function  
| ~~QPTjCyte Developing Cell-based RP  
Treatment at Ophthalmology  
Innovation Summit @ AAO jCyte~~



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#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

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~~Professor Robin Ali on gene and cell  
therapies to prevent blindness due to  
retinal disease~~ **3. Retinal Cell Fate  
Determination**

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ReNeuron Group - Gene & Cell  
Therapy Spotlight at OIS @ AAO 2019  
~~Retinal Gene Therapy 2018 at  
Ophthalmology Innovation Summit~~

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**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

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status and the successful clinical trials  
of RPE65 ...

~~Restoration of retinal and visual  
function following gene ...~~

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## ~~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 ...

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~~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

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Innovation Summit 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



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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.

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~~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...

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~~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.

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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

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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

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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.

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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~~

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~~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



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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

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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

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## ~~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

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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~~

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~~treatment for rare ...~~  
Gene expression of Nestin, paired box protein 6 (PAX6), Thy1 and brain-specific homeobox/POU domain protein 3 (Brn3) in retinal progenitor cells was detected by reverse transcription-quantitative polymerase chain reaction.

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~~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

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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-

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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



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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.

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~~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.

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~~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.

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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

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## 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.

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