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cell and gene therapy  
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what you in the same  
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Retinal repair:

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

Retinal Research

Update - 4/18/2020

~~Retinal regeneration~~  
~~and photoreceptor~~  
~~reprogramming My~~

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~~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?

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Gene Therapy for  
Inherited Retinal  
Diseases Clinical  
Embryology of The  
Eye - Part 1 (Basics)

Ophthotech:

Transformative Gene  
Therapy and Novel  
Therapeutics in  
Retina

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GenSight: Disruptive  
Gene Therapy in  
Retinal and  
Neurodegenerative

# Read Online 3rd Retinal Cell And Disease Therapy

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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



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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

# Read Online 3rd Retinal Cell And

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

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ReNeuron Group -  
Gene /u0026 Cell  
Therapy Spotlight at  
OIS @ AAO 2019  
~~Retinal Gene Therapy  
2018 at  
Ophthalmology  
Innovation Summit  
Retina - OIS~~  
Reversing Blindness  
with New FDA  
Approved Retinal  
Gene Therapy 3rd

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~~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~~

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~~following gene ...~~

3rd retinal cell and  
gene therapy  
innovation summit is  
available in our  
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Merely said, the 3rd  
retinal cell and gene

...

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Gene therapy

approaches to

treating inherited

retinal diseases are of

special interest given

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Gene Therapy  
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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

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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~~

*Page 16/39*



# Read Online 3rd Retinal Cell And 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

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Gene Therapy  
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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

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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

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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~~

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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

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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

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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

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## Innovation Summit

~~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



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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 ...

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~~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

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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

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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.

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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

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domain protein 3  
(Brn 3) in retinal  
progenitor cells was  
detected by reverse tr  
anscription quantit  
ative polymerase  
chain reaction.

~~Retinal ganglion cell-  
conditioned medium  
and surrounding ...~~

The gene, which is  
called RPE65, is  
injected into the eye,

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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 &~~

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~~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~~



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~~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-

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linked retinitis  
pigmentosa (RP),  
incurable genetic  
diseases that cause  
blindness in men.

~~Creation and spinout  
of Nightstar, a retinal  
gene therapy ...~~

Please use one of the  
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report: APA. Moore,

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Sarah. (2020, March 30). New method to treat blindness using retinal cell production.

~~New method to treat  
blindness using  
retinal cell  
production~~  
UK-based biotech  
ReNeuron has  
announced  
encouraging results

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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

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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~~

# Read Online 3rd Retinal Cell And 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.

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