



# My Journey with RP

Veronica McDougall

# Agenda

Meet Veronica

What is Usher Syndrome II & RP

Veronica's Timeline

Meet my Doctors

Getting Started

Content I, II & II

My Journey with RP

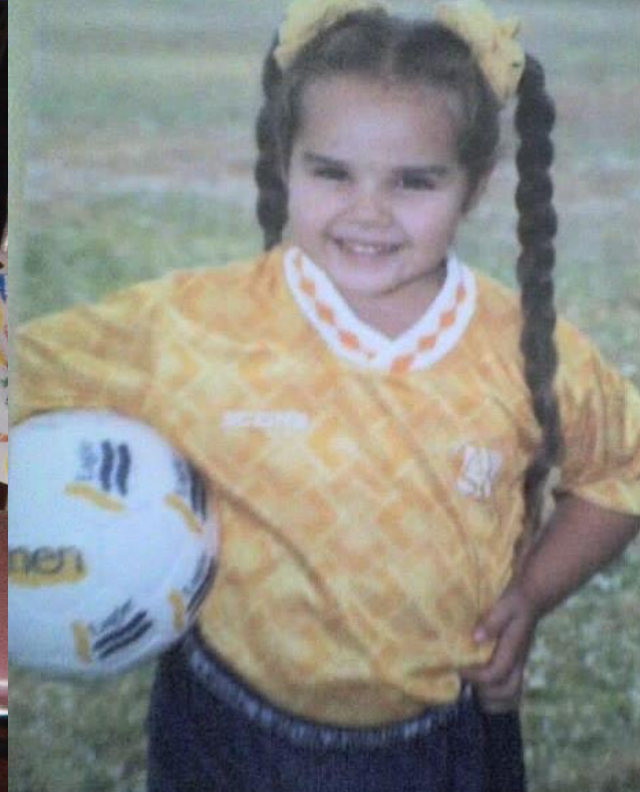
Meet my family

Meet Robert & Elliott

Thank you


Questions ?





# Meeting Veronica McDougall

- At the age of 3, my parents discovered that I was born hard of hearing. At that time, we were unaware that I would later be diagnosed with Usher Syndrome Type II, which has both hearing loss and Retinitis Pigmentosa.



Usher syndrome type II (USH2) is characterized by hearing loss from birth and progressive vision loss that begins in adolescence or adulthood. The hearing loss associated with this form of Usher syndrome ranges from mild to severe and mainly affects the ability to hear high-frequency sounds.

Retinitis pigmentosa (RP) is a progressive, bilateral, symmetric retinal degeneration that begins with night blindness and constricted visual fields (tunnel vision) and eventually includes decreased central visual acuity; the rate and degree of vision loss vary within and among families.

Also known as a chronic hereditary eye disease characterized by black pigmentation and gradual degeneration of the retina.

There are an estimated 1 in 3,500 to 1 in 4,000 people in Europe and the U.S. who have retinitis pigmentosa. Globally, RP affects about 1 in 3,000 to 1 in 4,000 people, or about two million people total. In the U.S., this total is estimated at about 100,000 people.

# Veronica's Timeline

Veronica diagnosed by  
Dr. Osman & Dr. Lyons  
2016

Veronica's  
Timeline

We researched for  
Retinitis Pigmentosa's  
Cure/Treatment  
Globally

Dr. Klassen & Dr. Yang  
Clinical Trial #1 & #2

Herbert Gavin  
Eye Institute

Assessment

Dr. Kuppermann  
assessed Veronica  
for Clinical Trial

Dr. Mehta injected stem cell  
treatment  
2/21/18 & 2/22/2021

Received Treatment

jCyte Testing

Dr. Kammer  
performs testing  
after injections

Waiting for 3<sup>rd</sup> Trial &  
FDA Approval

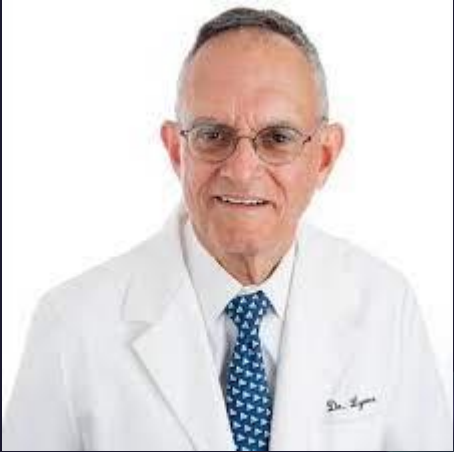
Future



# Meet my Doctors:



Michael H. Osman, M.D.  
Retina Specialist &  
Vitreoretinal Surgeon



Dr. Jonathan Lyons, MD  
Ophthalmologist



Baruch D. Kuppermann,  
M.D., Ph.D. is the Roger  
F. Steinert Professor,  
Chair of the  
Department of  
Ophthalmology, and  
Director of the Gavin  
Herbert Eye Institute at  
the University of  
California, Irvine.



Henry J. Klassen, MD, PhD  
Professor of Ophthalmology  
University of California, Irvine



Jing Yang, MD, PhD  
Associate Adjunct Professor,  
Ophthalmology School of Medicine



Mitul C. Mehta, MD  
Associate Clinical Professor,  
Ophthalmology, School of Medicine



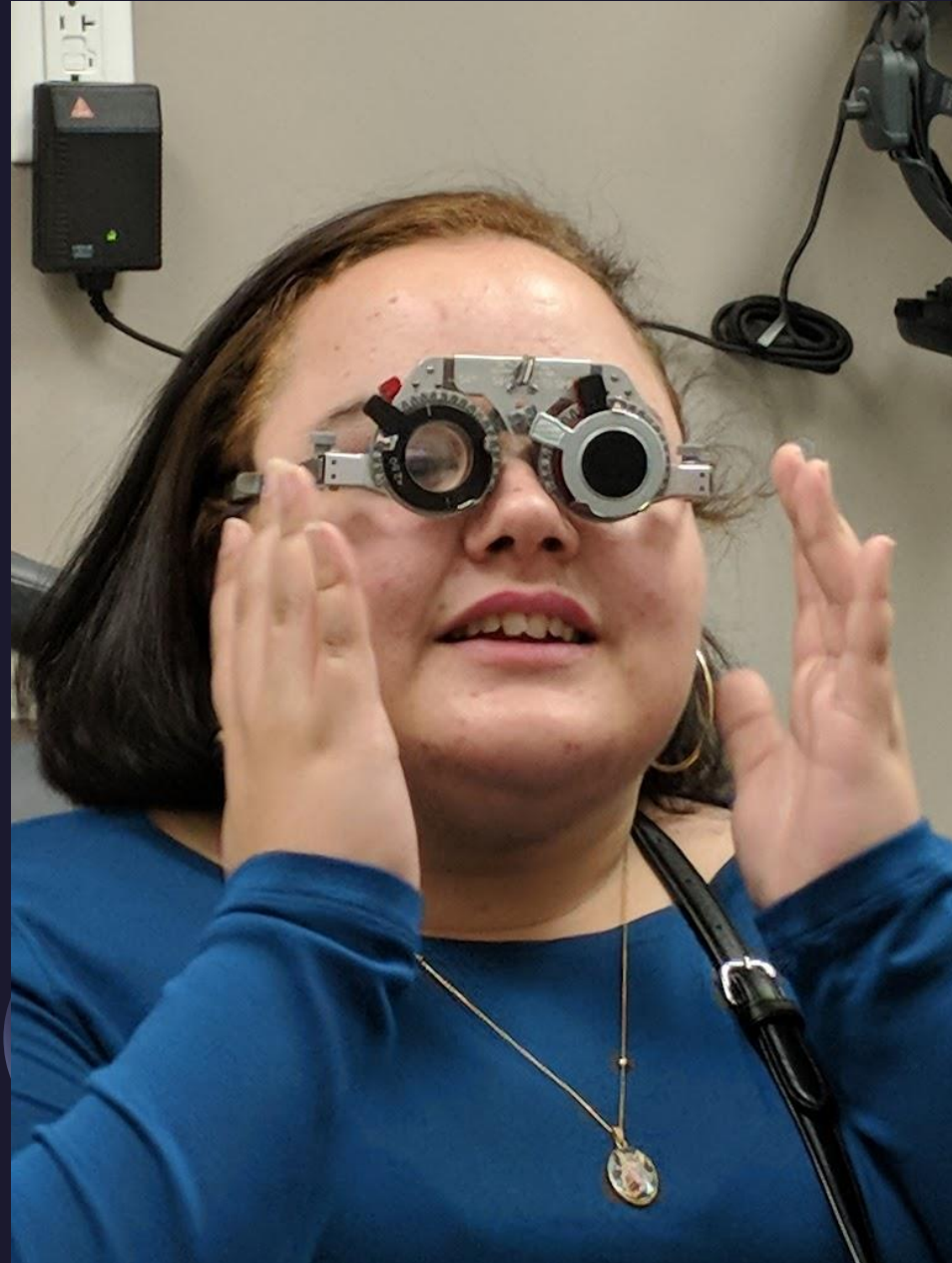
Dr. Rebecca Kammer  
Adjunct Professor, Ophthalmology  
School of Medicine

# The way to get started is to quit talking and begin doing.

Walt Disney.



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# Content One:

## IS RETINITIS PIGMENTOSA A RARE DISEASE?

Retinitis pigmentosa (RP) is a group of rare eye diseases that affect the retina (the light-sensitive layer of tissue in the back of the eye). RP makes cells in the retina break down slowly over time, causing vision loss. RP is a genetic disease that people are born with

## WHO IS MOST LIKELY TO GET RETINITIS PIGMENTOSA?

In most cases, the disorder is linked to a recessive gene, a gene that must be inherited from both parents in order to cause the disease.

But dominant genes and genes on the X chromosome also have been linked to retinitis pigmentosa

## AT WHAT AGE DO PEOPLE WITH RETINITIS PIGMENTOSA GO BLIND?

Retinitis pigmentosa usually starts in childhood. But exactly when it starts and how quickly it gets worse varies from person to person.

Most people with RP lose much of their sight by early adulthood. Then by age 40, they are often legally blind.

# Content Two:

## CAN RETINITIS PIGMENTOSA STOP PROGRESSING?

Retinitis pigmentosa often progresses gradually, but as it worsens, patients may suffer from night blindness, followed by a loss of side vision.

Unfortunately, there is no cure for retinitis pigmentosa.

## CAN I STILL DRIVE WITH RETINITIS PIGMENTOSA? RETINITIS PIGMENTOSA AND DRIVING

Since RP is a progressive condition that particularly affects peripheral vision, it's likely that at some point, your visual field won't meet the minimum standard.

Usually, your night vision will be affected first, meaning you will only be able to drive during the day.

# Content Three:

## HOW LONG DOES RETINITIS PIGMENTOSA TAKE TO PROGRESS?

Retinitis pigmentosa is a disabling disease that is currently incurable. It typically starts at the early teenage years and progresses to severe visual impairment during the 4th and the 5th decade.

## IS THERE A CURE COMING SOON FOR RETINITIS PIGMENTOSA?

Initial safety data from the PRODYGY study are expected in 2023. The FDA has cleared the investigational new drug application (IND) of SparingVision's gene therapy SPVN06 for treating retinitis pigmentosa (RP). Dec 4, 2022



# My Journey

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# Meet my Family



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Meet  
Robert  
& Elliott

# Thank You

Veronica McDougall

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# Questions ?

- What did you experience during your Clinical Stem Cell Trial?
- How has the Trial affected your daily life?
- How has this affected your ability to finish college?
- What dreams are you able to achieve?
- What are you doing to help Retinitis Pigmentosa's in the future?



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