GENE THERAPY’S JOURNEY FROM BENCH TO BEDSIDE:

FIRST GENE THERAPY FOR BLINDING EYE DISEASE GETS FDA APPROVAL

WINNING THE FIGHT AGAINST BLINDNESS
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KEEP YOUR EYES HEALTHY
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The FOUNDATION FIGHTING BLINDNESS
History was made on December 19, 2017 when the world’s first gene therapy for a blinding eye disease, Luxturna, received approval from the United States Food and Drug Administration (FDA). When the Foundation Fighting Blindness (FFB) was established in 1974, nothing was known about the genetics of blinding eye diseases. Thanks to FFB donors, our knowledge and capacity to treat these devastating diseases has grown exponentially.

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“Today’s approval marks another first in the field of gene therapy ... this milestone reinforces the potential of this breakthrough approach in treating a wide-range of challenging diseases.”

Scott Gottlieb, M.D.
FDA Commissioner
Inherited retinal diseases (IRDs) encompass a broad group of genetic retinal disorders, including Leber congenital amaurosis (LCA), retinitis pigmentosa (RP), Stargardt disease, and Usher syndrome. Although there are important differences between these diseases, they all involve the progressive loss of vision and are caused by mutations in any one of more than 220 different genes.

To determine if a gene therapy might work for you, it is essential to learn your genetic diagnosis. Luxturna, for example, is approved to treat patients with confirmed biallelic RPE65 mutation-associated eye disease (“biallelic” means that there are mutations in both copies of the gene). Some patients with this “genetic diagnosis” have LCA and others have RP. Luxturna works by delivering a normal copy of the RPE65 gene directly to retinal cells. These retinal cells then produce the normal protein that is needed to convert light into an electrical signal that can be interpreted by the brain. This normal protein is the “treatment” because it stops further vision loss and restores some functional vision.

Thanks to FFB donors, we are closer than we’ve ever been to new treatments! At the FFB, we will keep fighting blindness and funding research until there are accessible, innovative, sight-saving treatments for all Canadians.
Many blinding eye diseases are progressive, leading to deteriorating vision over time. These discoveries aim to stop vision loss before it leads to blindness.

There are no cures for blindness, which is why we are fighting to restore sight. These discoveries brought us closer to the final round: an end to blindness.

1 in 9 Canadians will experience irreversible vision loss before the age of 65. We can’t fight blindness without educating patients and the public on this epidemic.

Research alone won’t win the fight. Whether it’s new policies or better funding, we’re finding ways to put the products of research into the hands of Canadians.

Thanks to the support of Foundation Fighting Blindness donors, we had big wins to celebrate in 2017.

We brought the fight to four arenas with the greatest potential for impact.

HOW WILL WE WIN THE FIGHT AGAINST BLINDNESS?

ONE KNOCKOUT AT A TIME.

PRESERVE VISION

RESTORE SIGHT

EDUCATE

ADVOCATE
Saving photoreceptors

Photoreceptors are the eye’s light-sensing cells, which die off in many blinding eye diseases. Dr. Philippe Monnier advanced the fight to save these cells when he discovered a link between a unique protein in the human eye and photoreceptor death.

Combined drug therapy

In some genetic eye diseases, mutations lead to unfinished proteins that don’t work, resulting in vision loss. Dr. Cheryl Gregory-Evans discovered that the combination of two specific drugs has promising results for the fight to preserve vision.

New gene discovered

Dr. Michel Cayouette discovered a new “master regulator” gene involved in the production of photoreceptors. The discovery brings us one step closer to a stem cell therapy that restores vision by replacing damaged photoreceptors with healthy ones.

Restoring the macula

Loss of central (macular) vision is common across many eye diseases. Dr. Gilbert Bernier produced a macula-like retinal tissue using stem cells. The new tissue can be used for transplantation as well as the study of eye diseases – a double punch!

Community

We hosted 9 Vision Quest education events across Canada, which brought together more than 860 stakeholders and attracted more than 55,000 views of our interactive online sessions.

Stem cell tourism

Dubious stem cell clinics are offering dangerous “cures.” Our multimedia response saved the vision of many who were considering this perilous route.

Patient Registry

We expanded the reach of this tool, which connects patients to clinical trials, by planning a new enrollment site in Québec with the leadership of Dr. Robert Koenekoop.

Genetic discrimination

We helped push Bill S-201 through Parliament, ensuring companies can’t access your genetic history.

Historic gene therapy win

A US biotech firm received the first FDA approval for a gene therapy. Our advocacy helped pave the way. (see pages 1 – 2)

Government consultation

We worked with the Ontario Ministry of Health to put safety first when choosing new drugs for eye diseases.
THREE ACTIONS TO KEEP YOUR EYES HEALTHY IN 2018

While FFB-funded scientists are working to develop new sight-saving treatments, there are many things that you can do to take care of your vision health today. **In 2018, we challenge you to join us in making these 3 vision health resolutions.**

**GET AN EYE EXAM**
When vision deteriorates in only one eye, the eye with better vision will compensate for the poor eye. As a result, many eye diseases go undetected. This year, make a plan to see your optometrist or ophthalmologist to ensure that there are no early signs of cataracts, glaucoma, or macular degeneration. Early detection is essential to ensure proper treatment!

**GET COLORFUL**
To promote healthy vision, add colour to your diet! Your ideal plate should include spinach, kale, carrots, and red peppers with almonds on top. This colourful salad is full of vitamin A, E, and C. Leafy greens, such as kale and spinach, are also chock-full of lutein and zeaxanthin, which are food-derived nutrients that help protect your eyes. Carrots won’t give you the eyesight of a superhero, but they do contain enough beta-carotene to contribute to healthier eyes. Beta-carotene is used by the body to produce vitamin A, which is essential to eyesight.

**GET MOVING**
Exercise is good for your heart, good for your eyes, and good for your mind! Exercise can help your vision health by reducing inflammation in your body, including your eyes. We all know that exercising can be challenging, especially in the winter. Don’t let yourself get overwhelmed by the idea of breaking a sweat in a gym. Just make more choices to move around—you might even consider joining FFB’s cycling fundraising event: Cycle for Sight!
Being young and being philanthropic don’t often go together, and for good reason. Young people, especially those between 17 and 30, face challenges that are both daunting and unprecedented: tuition fees are inflated, job markets are increasingly constrained, and home ownership is often beyond reach. These challenges are heightened for those living with vision loss and blindness.

None of this has stopped Anson Wu from fighting for what he believes in. Diagnosed with retinitis pigmentosa (RP) at 7-years-old, Anson would face the hurdles that so many with RP and other retinal diseases experience. Due to the loss of his peripheral and night vision, many activities became inaccessible and friends were hard to come by. However, Anson persevered and continued to work hard in both academics and sports. In high school, he was placed in the gifted program and won many academic awards. He also played an active role on the school’s swim team, culminating with a back to back 50m and 100m freestyle gold in the York Regional Championships and team MVP honours. Anson went on to study science at the University of Guelph, earning his Masters in bioinformatic,a field that combines computational science and statistics to analyze complex biological data.

Now 27-years-old, Anson has been donating monthly to the Foundation Fighting Blindness for 10 years, proving that youth and philanthropy are not mutually exclusive. He supports vision research because he believes in a future where vision can be restored and vision loss reversed. In the meantime, he lives according to the belief that you should “adapt to what you have and never give up,” regardless of the challenges you face. Thank you, Anson, for your generous support, and for showing us that no obstacle is insurmountable.

Anson Wu has been a monthly donor for 10 years.

>> LEAVE A LEGACY
Support vision research by leaving the Foundation Fighting Blindness in your will.

>> DONATE SECURITIES
A gift of stock can maximize the value of your donation – and your tax advantage.

For more information on these or other ways to make a gift please contact Ann Morrison 1.800.461.3331 ext. 232
GET INVOLVED
HELP END BLINDNESS

From participating in one of our three signature events, to creating and hosting your own unique fundraiser, we encourage anyone interested in supporting research to get involved and help change the future for individuals and families living with vision loss.

WANT TO HOST YOUR OWN EVENT?
Check out our Sight-Savers page at ffbc.ca to learn more or contact us at 1-800-461-3331.