#### Disclaimer March 2023

Robert's extract conversations with ChatGPT, AI, relating to his online posting about his "wet macular degeneration".

Always consult your professional registered health specialists, if in any doubt. The evaluation of the merit, accuracy, bias and limitations of the AI responses to my questions, is left to the individual reader.

This PDF document is to be read in conjunction with my online posting.

Caveat emptor applies.

\_\_\_\_\_\_

6

# Regenerative medicine ~ future possible treatments for eye diseases.

Gene and stem cell therapies and CRISPR. Guesstimate future timelines for possible cures.



Continue: in the context of current developments, seeking a cure for eye diseases causing severe vision loss or blindness, differentiate between the following: gene therapy, CRISPR and stem cell therapy. Give evidence if any, if these procedures actually currently restore vision loss.

Gene therapy, CRISPR, and stem cell therapy are all promising approaches in the field of regenerative medicine for treating eye diseases causing severe vision loss or blindness. However, they differ in their methods and mechanisms.

Gene therapy involves replacing or repairing defective genes responsible for a particular disease. In the context of eye diseases, this could involve introducing a healthy copy of a gene into the cells of the retina to restore or improve vision. There have been some successful clinical trials using gene therapy to treat certain inherited retinal disorders, such as Leber congenital amaurosis (LCA) and choroideremia. While these trials have shown some improvement in vision, they are still in the early stages and have not yet been approved for widespread use.

CRISPR is a gene-editing tool that allows scientists to make precise edits to the DNA sequence of a cell. In the context of eye diseases,

CRISPR could be used to correct or remove mutations in genes associated with vision loss. There have been some successful preclinical studies using CRISPR to treat inherited retinal disorders, such as retinitis pigmentosa and Usher syndrome. However, the use of CRISPR in humans is still in the early stages of development and has not yet been approved for clinical use.

Stem cell therapy involves using stem cells to replace or regenerate damaged cells in the retina. The idea is to transplant healthy cells derived from stem cells into the damaged retina to restore or improve vision. There have been some successful preclinical studies using stem cell therapy to treat age-related macular degeneration and Stargardt disease. However, the use of stem cell therapy in humans is still in the early stages of development and has not yet been approved for widespread use.

Overall, while there have been promising results from these approaches, they are still in the early stages of development and have not yet been approved for widespread use to restore vision loss.



Continue: in particular regard to gene therapy only, is this therapy only a preventative therapy to stop the occurrence of an eye disease, or is this therapy that will restore existing severe loss of vision or blindness in a person.

Gene therapy for eye diseases can have both preventative and restorative capabilities. In some cases, it can be used to prevent the progression of a disease that could potentially lead to severe vision loss or blindness. However, there are also ongoing clinical trials investigating gene therapy as a potential treatment for individuals who have already experienced significant vision loss or blindness. One example is a clinical trial for a gene therapy called Luxturna, which was approved by the US Food and Drug Administration in 2017 for the treatment of an inherited form of vision loss. This therapy has been shown to improve vision in some individuals who were previously blind or had severe vision loss due to this genetic condition.

### ?

Continue: in reality, how far away is the actual use of these aforesaid therapies to restore full sight to persons who have existing severe vision loss or are blind. Give your estimate in years, that these procedures will become normal practice to effectively restore major vision loss in a person.

As of now, these therapies are still in the experimental stage and have not been approved for general use. While some early-stage clinical trials have shown promising results, there is still a long way to go before these therapies can be used on a widespread basis to restore vision loss in people.

It's difficult to predict exactly how long it will take for these therapies to become a normal practice for restoring major vision loss in people. The timeline will depend on various factors, such as regulatory approval, availability of funding for research, and the success of clinical trials.

However, experts in the field estimate that it could take at least another decade or more before these therapies are widely available for use in restoring vision loss in people. This estimate is based on the current pace of research and the time required for conducting clinical trials, gaining regulatory approval, and establishing the necessary infrastructure for administering these therapies.



Continue: regarding our discussion about gene therapy, where does a healthy copy of a gene, which is introduced into the cells of the retina, come from. And how is it introduced into the retina cells.

A healthy copy of a gene for gene therapy can come from several sources, including a person's own cells, a donor's cells, or synthetic copies of the gene. In some cases, a healthy copy of the gene can be introduced into the cells of the retina using a harmless virus that has been modified to carry the gene. This virus is known as a viral vector and can deliver the healthy gene to the appropriate cells in the retina, where it can replace the faulty gene and potentially restore vision. Other methods for delivering the healthy gene include using nanoparticles or electric fields. However, it is important to note that gene therapy for

vision loss is still in the early stages of development and has yet to be widely implemented as a treatment option.



Continue: I have no evidence that this treatment option is available anywhere today. Your reply please.

I apologise for any confusion. Currently, gene therapy for inherited retinal diseases is still in the experimental stage and not yet widely available. There have been some successful clinical trials, but more research is needed before this treatment becomes a standard practice.



Continue: give us your best estimate for how many decades will pass before this procedure becomes standard.

As I mentioned earlier, it's difficult to give an exact estimate as it depends on various factors such as the success of ongoing research, clinical trials, regulatory approvals, and accessibility to the treatment. However, based on the current pace of research and clinical trials, it is possible that gene therapy for restoring vision loss may become a standard treatment option within the next few decades. Some experts even speculate that it may become a reality within the next 10 to 20 years, but it's important to note that this is just a speculation and not a guarantee.



Continue: regarding stem cell therapy: again where do healthy stem cells come from which are transplanted into the retina. How does this transplant take place. And, how long does it take to regenerate the new retinal component, which results in the possible restoration of sight. Please explain.

Stem cells used for retinal regeneration can come from various sources, including embryonic stem cells, induced pluripotent stem cells (iPSCs),

and adult stem cells. These cells can be harvested from a variety of sources, such as bone marrow or adipose tissue, and then grown in a lab under specific conditions to become retinal cells.

The transplant of stem cells into the retina can be done in different ways. One method is to inject the cells into the vitreous humor, which is the jelly-like substance that fills the back of the eye. Another method is to transplant a layer of retinal pigment epithelium (RPE) cells, which support the photoreceptor cells in the retina, into the back of the eye.

The time it takes to regenerate new retinal tissue and potentially restore vision can vary depending on several factors, such as the type and severity of the retinal disease, the age and overall health of the patient, and the effectiveness of the treatment. Some studies have shown promising results in animal models and early clinical trials, but further research is needed to determine the safety and long-term effectiveness of this approach in humans.

As for the timeline for the widespread use of stem cell therapy for restoring vision in humans, it is difficult to predict. While there have been some successful early trials and promising results in animal studies, more research is needed to ensure the safety and efficacy of the treatment. It could take several more years, if not decades, for this therapy to become widely available to the general public.

?

Continue: again, it clearly seems that the use of stem cell therapy for the restoration of sight in humans is actually a very long way off in the future. Give your opinion as to how many decades must pass before this becomes a broad-based welcomed cure. Your best timeline please.

As an Al language model, I cannot predict the future with certainty, but based on current research and clinical trials, it is likely that it will take several decades before stem cell therapy becomes a widely accepted and effective treatment for severe vision loss or blindness. The technology is still in the early stages of development and clinical trials are ongoing. It may take several years or even decades before the technology is refined and made widely available to patients. Additionally, regulatory approval and funding for research and development will also play a role in determining the timeline for widespread adoption.

## ?

Continue: in regards to CRISPR, explain how this gene-editing tool actually works to make precise edits to a DNA sequence of a cell. How is this incorporated into the retina.

CRISPR is a revolutionary gene-editing tool that has the ability to make precise edits to the DNA sequence of a cell. It stands for "Clustered Regularly Interspaced Short Palindromic Repeats" and is based on the natural defence mechanisms of bacteria against viruses.

CRISPR works by using an enzyme called Cas9, which is guided to a specific location in the DNA by a molecule called a guide RNA. Once the Cas9 enzyme is targeted to a specific site in the DNA, it cuts the DNA at that location. This cutting of the DNA can trigger the cell's natural repair mechanisms to repair the break in the DNA. Researchers can use this process to add or delete specific genes from the DNA sequence.

To incorporate CRISPR into the retina, researchers are exploring different methods such as using a viral vector to deliver the CRISPR components to the target cells in the retina. This delivery mechanism needs to be highly specific to ensure that only the target cells are edited and not other cells in the body.

However, it's important to note that CRISPR is still in the experimental stage and its safety and efficacy in human trials are still being evaluated. While there have been successful demonstrations of CRISPR being used to edit genes in the retina of mice, it will take time to determine if it will be effective in humans.

### ?

Continue: Again, give your best evaluation estimate of when CRISPR will be in wide spread use to address issues of severe vision loss or blindness in humans. Your estimate in decades please.

As with any new technology or medical treatment, the timeline for widespread use can be difficult to predict. There are a number of factors that can influence the speed at which a new technology is adopted,

including regulatory approval, cost, and the complexity of the procedure itself. While there has been some promising research on the use of CRISPR for vision loss and blindness, it is still in the early stages of development and more research is needed to determine its safety and effectiveness.

Therefore, it is difficult to provide a precise timeline for when CRISPR will be in widespread use for addressing severe vision loss or blindness in humans. However, some experts predict that it may take at least a decade or more before the technology is widely available for clinical use. It is important to note that any new treatment must go through rigorous testing and clinical trials to ensure its safety and efficacy, which can add to the timeline.

#### ?

Continue: with respect to gene and stem cell therapies and CRISPR, I have formed the view that these current new medical technologies are simply decades away for providing a cure for vision loss or blindness in the human population. Please discuss. Are there any other new medical technologies out there seeking cures for vision loss and blindness.

While it is true that gene and stem cell therapies, as well as CRISPR, are still in the early stages of development and it may take several decades before they become widely available treatments for severe vision loss or blindness, there are other new medical technologies that are being developed to address these issues.

One promising area of research is optogenetics, which involves using light-sensitive proteins to control the activity of nerve cells in the retina. This approach has shown some success in restoring vision in animal studies and is currently being tested in clinical trials.

Another area of research is the use of artificial intelligence and machine learning to develop new diagnostic tools and treatments for vision loss. For example, researchers are using machine learning algorithms to analyse images of the retina to detect early signs of disease and predict disease progression.

In addition, there are also new prosthetic devices and implantable technologies being developed that can restore some degree of vision to

people with severe vision loss or blindness. For example, retinal implants are being used to stimulate the remaining cells in the retina to create visual signals that can be sent to the brain.

While it may still take several decades before a cure for severe vision loss or blindness is found, these new technologies offer hope for people with these conditions and are paving the way for new treatments and improved quality of life for those affected.