

Gene therapy for wet age-related macular degeneration (wet AMD)

A single anti-VEGF treatment with the potential for long-lasting results



Learn about the clinical trial for people with wet AMD.

What is wet AMD?

Wet age-related macular degeneration, or wet AMD, is a progressive eye condition affecting older adults.

Wet AMD is the leading cause of blindness in the United States and can interfere with the ability to:



Read



Write



**Recognize
faces**



Drive



**Do other
everyday
activities**

In individuals affected by wet AMD, new blood vessels form in the retina, a part of the eye responsible for sight. These new blood vessels don't work as they should, leaking blood and fluid and causing damage to the retina. The formation of these vessels can be caused by the eye releasing a protein called vascular endothelial growth factor (VEGF).

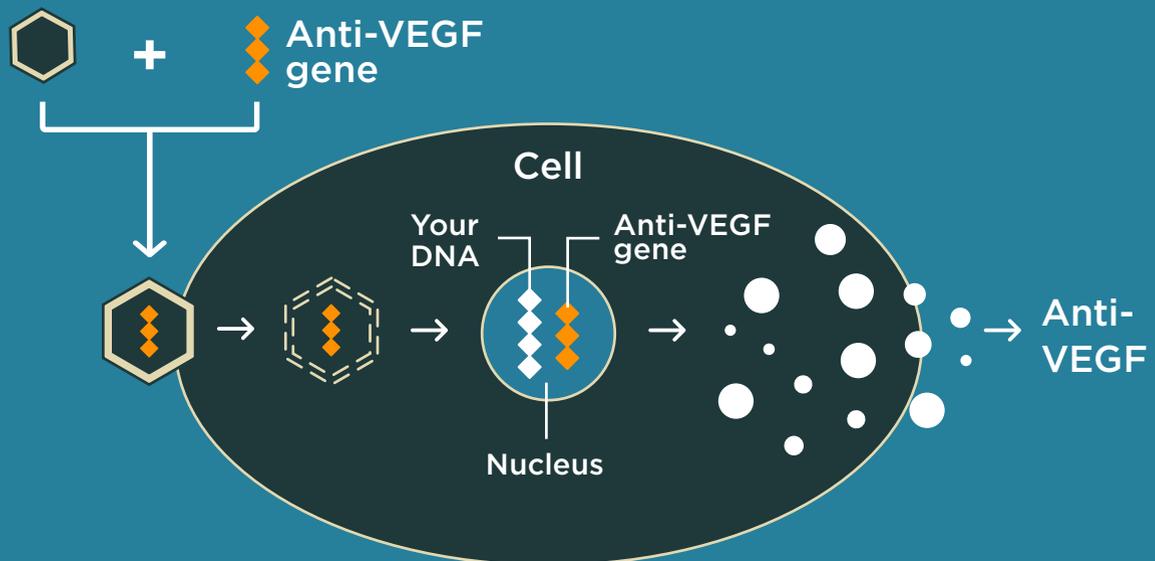
People with wet AMD need long-term treatment to try to slow the growth of these new blood vessels and the damage they cause. The condition is currently treated with anti-VEGF therapy, which involves an injection into the eye. It requires frequent visits to the doctor to get the injections and can be a significant burden.

What is gene therapy?

Gene therapy is a way of placing copies of a gene into cells in order to allow the cells to produce proteins that may treat a condition.

- The gene is delivered to the cells using a modified virus, or **vector**, that has been used previously in humans and is not known to cause disease in humans
- The vector is designed to **deliver the gene** to specific cells in the body
- Once the gene is delivered to the cells, the cells may be able to produce the **anti-VEGF protein** on their own
- This potentially **one-time therapy** may produce long-lasting results and could replace frequent anti-VEGF injections

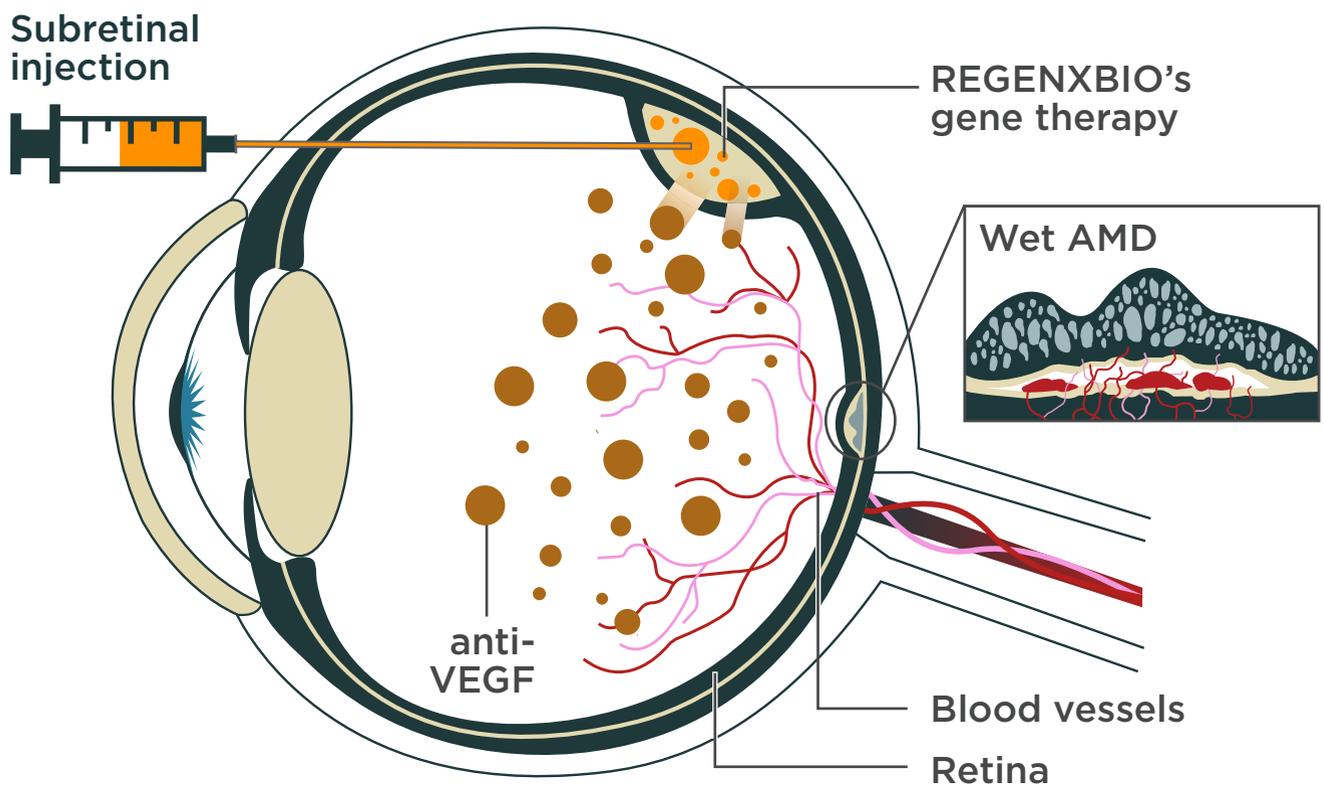
Vector



How does gene therapy for wet AMD work?

REGENXBIO's gene therapy (RGX-314) for wet AMD is designed to deliver a gene that sits inside the cells of the retina (the gene does not become a part of the cell's DNA). Rather than requiring regular injections of anti-VEGF, **the gene is injected once into the eye**, under the retina (called subretinal injection), potentially allowing the cells in the retina to make their own anti-VEGF. This may decrease the need for future treatment for wet AMD and help prevent further vision loss.

The gene therapy is performed at a study center and is a surgical procedure that can usually be done on an outpatient basis.



Clinical trial details

Safety

This clinical trial is a Phase I trial, meaning that this is the first time REGENXBIO's gene therapy is being tested in humans to treat wet AMD. The study is designed to test the safety and tolerability of the gene therapy. It has been shown to be safe and effective in mice and monkeys.

What's involved?

Participants will need to visit the study center multiple times during the first month of the study and monthly thereafter for up to 2 years following the therapy. Participants will be encouraged to participate in long-term follow-up visits as well. These visits will be important because they will provide valuable information to researchers. Transportation to the study center will be provided free of charge, and compensation for each study visit will be given. Additionally, all study-related testing and anti-VEGF injections, if needed, will be provided.

Learn more

To learn more about REGENXBIO, the sponsor of this study, and its gene therapy development, visit [REGENXBIO.com](https://www.REGENXBIO.com) or contact patientadvocacy@REGENXBIO.com.

Participation

Talk to your doctor if you or a loved one is interested in this gene therapy study.



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