

Hope for new treatments buoys macular degeneration patients

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Craig F. Walker/Globe Staff

Althea Lank is treated for age-related macular degeneration at Mass Eye and Ear Infirmary.

Sometimes it starts with wavy vision. Objects appear distorted. Familiar faces go blurry.

Sean Teare, a 48-year-old health care consultant from Duxbury, struggled to read menus in dimly lit restaurants. After a battery of tests, his optometrist told him he had age-related macular degeneration, or AMD, an eye disease that afflicts more than 9 million Americans and can cause serious vision loss. “It came as a complete shock,” said Teare.

The prevalence of the condition is rising as the population ages. The number of early-stage cases for those 50 and older is projected to nearly double to 17.8 million in the United States by 2050, according to the Centers for Disease Control and Prevention. For baby boomers, who are living longer than past

generations and fiercely prize their independence, it's a dreaded diagnosis that threatens to rob them of everyday functions such as reading, driving, cooking, or watching television.

With the increase in cases has come a burst of research activity. There's currently no cure for the disorder, and no treatment for its most common form, which accounts for 85 percent of cases.

But scientists in Massachusetts and around the world are experimenting with dozens of drug candidates, including about 20 in clinical trials, that work to preserve vision and, ideally, restore sight. They include not only well-established drugs, such as repurposed statins, but also new approaches such as gene therapies, stem cell treatments, and medicines tailored to the genetic makeup of patients.

"We're close to seeing some important findings," said Dr. Joan Miller, chief of the ophthalmology department at Massachusetts Eye and Ear Infirmary in Boston.



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Dr. Joan Miller spoke with Lank before treating her for age-related macular degeneration at Mass. Eye and Ear last week.

The disease, considered the leading cause of blindness in older Americans, is triggered by fatty deposits that damage a spot in the retina called the macular, which lets the eye see fine detail. Its rate of progression varies. Some patients don't experience vision loss for many years; others lose sight in their central field of vision, inhibiting their ability to see straight ahead, but retain peripheral vision.

Patients with a more severe form of the disease can receive periodic injections of an antibody into the eye that can slow progression of the disease by blocking leaky blood vessels.

Miller, who helped pioneer the science behind Lucentis, approved by the Food and Drug Administration in 2006 as the first treatment for age-related macular degeneration, hopes to see a new generation of treatments emerging in the next five to 10 years.

Some of those new treatments could be less uncomfortable and time-consuming than today's injections. The emerging field of gene therapies, for instance, promises "one and done" procedures that could stop the disease in its tracks by inserting healthy genes into cells in place of defective or missing ones. Another approach involves stimulating cells in the retina to act as mini-production factories that generate proteins to protect the macular.

Such advances can't come soon enough for such patients as Laura Brennan, 64, of South Boston, who gets shots into her eyes every two months to stabilize her vision.

Brennan, who first experienced wavy vision when she was in her 50s, is determined to keep living her normal life. The injections and other adjustments have enabled her to continue walking, swimming, and working as a chef for Foodie's Markets in South Boston and the South End.

"When I first noticed that I couldn't make out someone's face across the room, that was very difficult," said Brennan, who recalls her father also developing macular degeneration late in life. "But I've been able to adapt. I know who people are by their steps or their voice. At this point, my goal is to preserve the vision that I have, not to have it decrease anymore."

Hemera Biosciences, a Waltham startup, is seeking to develop a kind of vaccine that would make treatments easier and less invasive for patients like Brennan.

“Patients in their 60s and 70s will go to their ophthalmologists,” said Hemera chief executive Adam Rogers. “If they’re diagnosed with AMD, they can receive a shot and keep it at bay during their lifetimes. I think that’s something we could see in the next five to seven years.”

Biopharma giants such as Genentech, Novartis, and Regeneron are also working on experimental medicines. So are a raft of biotech startups ranging from Cambridge’s Gemini Therapeutics to Regenxbio in Rockville, Md.

For drug makers, the tens of millions of people with age-related macular degeneration are a potentially lucrative market. Sales of current medicines, mostly first-generation treatments including Lucentis, totaled nearly \$5 billion in 2016, and the expected new drugs will expand the market to \$11.5 billion by 2026, the British analytics firm GlobalData projects.

The approval of the first-ever gene therapy for any disease last December galvanized eye researchers. The new drug, Luxturna, treats a rare genetic retinal disease in children by replacing a mutation with a corrective gene. In March, Mass Eye and Ear performed the first-ever procedure to administer the drug to a patient.

“It opened up the avenue for other gene-based treatments, and some of that might be applicable to AMD,” said Miller.

While macular degeneration is thought to be influenced not only by genetics but by environmental factors, such as smoking, “gene therapies have incredible potential” to treat the disease, said Luk Vandenberghe, cofounder of Odylia Therapeutics, a Boston nonprofit working to commercialize retinal disease research. Decades of research to understand diseases is now helping to power the new approaches to treatments, he said.

There’s also hope that the success of gene therapies for maladies of the eye could help launch similar kinds of treatments for other diseases.

Ben Shaberman, an official at the Foundation Fighting Blindness, a patient advocacy group, said the retina — a thin tissue lining the back of the eye — is emerging as an ideal proving ground for the young gene therapy field.

“The retina’s accessible and a really good target,” he said. “If you get things to work in the retina, there’s a good chance you could apply them to neurodegenerative disorders of the brain or the central nervous system.”

Gemini, based in Kendall Square, is trying to bring the precision medicine model being deployed in targeted cancer treatments to AMD. Unlike drug developers that try to make one-size-fits-all treatments for macular degeneration, it's focusing on treatments tailored to subsets of patients with distinct genetic variations that put them at risk.

“We believe that genetics plays a key role, and we’re spending a lot of time trying to understand these subpopulations,” said Gemini chief executive James McLaughlin.



MARATHONFOTO

Sean Teare, a 48-year-old who has age-related macular degeneration, ran the Marine Corps Marathon last year.

Sometimes patients themselves aren't sure what role genetics has played in their disease. Teare, who was diagnosed with the disease in 2016, doesn't know anyone in his family with it. He wonders if his exposure to sunlight while boating or skiing was a factor.

Teare feels lucky to have the less severe form of the disease. And he's been quick to embrace lifestyle changes — eating a diet rich in fish and vegetables and wearing sunglasses with ultraviolet eye protection — in an effort to keep it from progressing. Last year, he ran the Marine Corps Marathon in Washington, D.C., to raise money for the Foundation Fighting Blindness.

He's counting on his healthy diet and lifestyle — and his upbeat attitude — as he awaits the progress of research programs.

“This isn't a terminal illness,” he said. “I feel I can make lifestyle changes that will keep it from progressing until there's some kind of treatment.”

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