# First Hints That Stem Cells Can Help Patients Get Better

by Rob Stein All Things Considered / January 23, 2012

Melissa Forsyth for NPR

Sue Freeman, 78, washes Brussels sprouts at her home in Laguna Beach, Calif., on Saturday. Prior to getting an experimental stem-cell procedure last July, Freeman couldn't cook, read or recognize faces.

Two women losing their sight to progressive forms of blindness may have regained some vision while participating in an experiment testing a treatment made from human embryonic stem cells, researchers reported today.

The report marks the first time that scientists have produced direct evidence that human embryonic stem cells may have helped a patient. The cells had only previously been tested in the laboratory or in animals.

"I can't tell you how excited I am about this," said Steven D. Schwartz, a professor of ophthalmology at UCLA's Jules Stein Eye Institute leading the research. "For these patients, the impact is enormous."

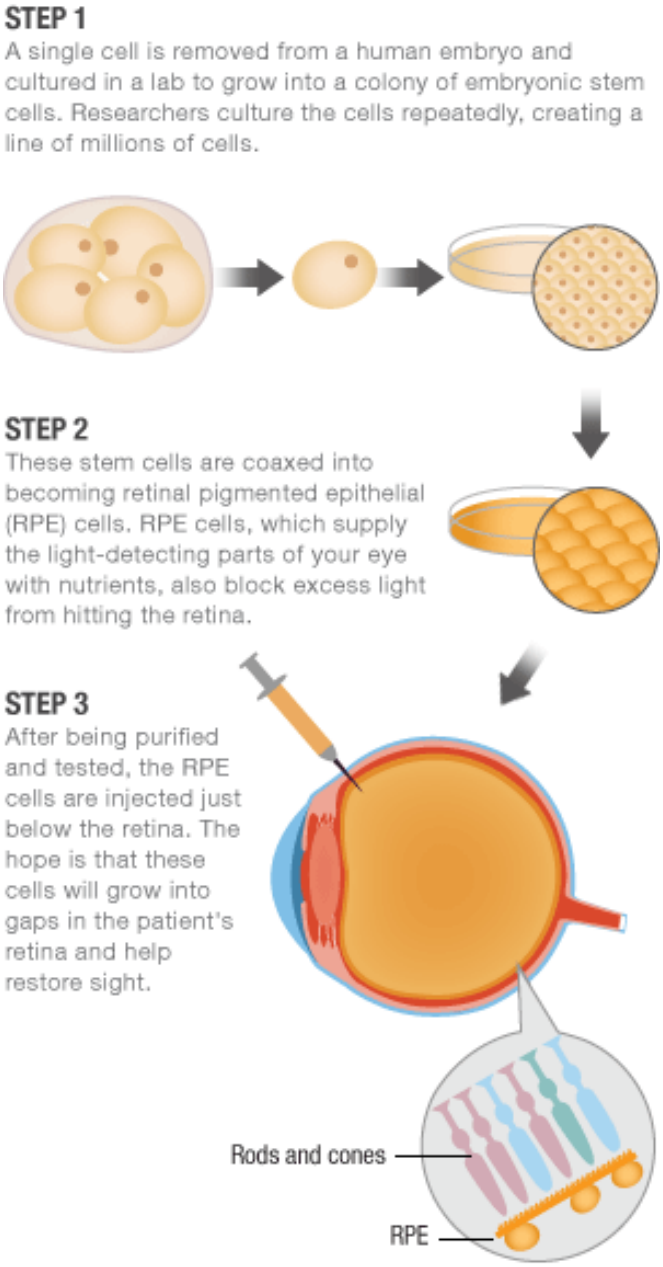
Schwartz and his colleagues stressed that the findings are extremely preliminary and it's far too early to know anything for sure. The patients could continue to improve, or their vision could deteriorate again, he said. Many more patients will be needed to be treated for far longer to know whether the therapy is really safe and responsible for any improvement.

"My job is to decrease suffering, and if we overstate this and raise hopes falsely and then it doesn't work out, it will hurt people rather than help them," Schwartz said.

But the findings could mark an important milestone for the field of human embryonic stem cells, and so-called regenerative medicine, Schwartz and others said.

"In the landmark paper by Schwartz and colleagues, the potential to use human embryonic derived cells with a therapeutic effect in patients is now finally realized," wrote Anthony Atala of the Wake Forest Insitute of the Wake Forest School of Medicine in a commentary accompanying the report in the journal The Lancet.

## How The Treatment Works




Source: Irina Klimanskaya / Advanced Cell Technology / Credit: Nelson Hsu, Melanie Taube, Julia Ro / NPR

Scientists discovered human embryonic stem cells in 1998. Many researchers believe they could revolutionize medicine because they can morph into virtually any type of cell in the body. That means they could potentially provide cells to treat many diseases, including diabetes, Alzheimer's, Parkinson's and spinal cord injuries.

But the field has been the focus of intense debate and controversy because human embryos are often destroyed to obtain the cells. Critics consider any research on human embryos, and especially the destruction of human embryos, to be immoral.

The Food and Drug Administration has only approved two studies testing any therapies made from human embryonic stem cells in people. The first involved patients who were partially paralyzed by spinal cord injuries. But that experiment was discontinued soon after it began last year when Geron, the company sponsoring the research, announced it was diverting funds to other projects.

The blindness study, which is being sponsored by Advanced Cell Technology, will involve 24 patients suffering from Stargardt's macular dystrophy, which is the leading cause of pediatric blindness, and dry age-related macular degeneration, which is the leading cause of blindness in the developed world.

Scientists used embryonic stem cells to create retinal pigment epithelium cells in the laboratory. RPE cells are lost in a variety of eye conditions, including Stargardt's and macular degeneration.

In July, Schwartz and his colleagues injected about 50,000 RPE cells made from stem cells into the right eye of Sue Freeman, 78, of Laguna Beach, Calif., who had lost most of her vision to macular degeneration.

Because of the disease, she could no longer recognize faces, read, cook or even go outside on her own. But within six weeks of the procedure, Freeman started to notice she could see landscapes better. Tests showed she could read more letters on an eye chart. Soon, she was making her own breakfast again and even has gone shopping alone.

"One day, I looked down and I could see my watch," she said. "I probably hadn't seen it in about a year and a half or two. And I could see. So that was exciting for me. And I remember saying, 'Oh my goodness. I can see my watch. I can actually tell time.' "

Schwartz and his colleagues are somewhat baffled by Freeman's improvement, especially since she initially reported being able to see better with both eyes, including the one that wasn't treated.

That made them suspect that something else may be causing the seeming improvement, such as the anti-rejection drugs she was taking or the placebo effect. But they have since become more confident her improvement is indeed being caused by the cells.

They are even more confident about the improvement by the second patient, who asked not to be identified to protect her privacy. She's a woman in her 50s who works as a graphic artist and started losing her sight in her 20s because of Stargardt's. When doctors examine her eye, they can actually see the transplanted cells thriving.

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Sue Freeman at her home in Laguna Beach, Calif. "It was pretty amazing," she says of the improvement in her vision. "I was like kind of looking at everything new again, just sort of going around and first not believing it."

"I sort of like woke up one morning and did realize that, 'Wow, you know, there is a difference between the two eyes now — they only worked on the left eye," she said. "On the other side of the room I have some hand-carved furniture there. And I could actually see the detail on the carving, you know, on the other side of the room there, on things that I couldn't see from that distance before."

She has since begun working much more easily, doing routine chores around the house and even riding a bike again.

"It was pretty amazing. I was like kind of looking at everything new again, just sort of going around and first not believing it but then really looking and, you know, realizing that I definitely had more sight in that eye," she said.

Now, Schwartz and his colleagues stress that the study was designed primarily to determine whether the cells were safe, and not to determine whether they might actually help patients. For this study, patients received very low doses, and their vision had been so damaged that no one thought the cells could help them.

Schwartz treated a third patient on Tuesday, and doctors in London began treating Stargardt's patients there on behalf of the company. Much more research is needed, they stress.

"It's just far too early to make any conclusions," said Robert Lanza, the company's chief scientific officer.

But if the findings are confirmed, Lanza said they would mark an important, long-awaited step.

"I think this is a turning point. It's been 13 years since the discovery of human embryonic stem cells," Lanza said. "We've been reading about this. It's been one of the hottest topics of biology. And this is the first report of the effects of these cells actually transplanted into a human patient. So it's been a long time in the works."