

Stem-Cell Therapy Is More Available, and Faces Scrutiny

By MELINDA BECK

In two days of hearings next month, the U.S. Food and Drug Administration will consider if clinics offering stem-cell treatments should be more closely regulated. Stem-cell treatments aren't approved by the FDA and not long ago, Americans had to travel to Mexico, China or elsewhere to receive them. Now, with the regulatory environment murky, clinics offering them are spreading rapidly across the U.S.

A recent report in the journal Cell Stem Cell counted 570 clinics advertising stem-cell therapies directly to consumers. Many claim to treat a long list of disorders, from arthritis to Alzheimer's disease, even though the stem-cell treatment for many of the conditions hasn't yet been tested on humans. Treatment typically costs thousands of dollars.

Critics, including many top stem-cell scientists, say they are peddling 21st century snake oil and want the FDA to crack down. Clinic operators say they don't need FDA approval because they are practicing medicine, not creating new drugs. Some patients say they have been helped and that the government shouldn't regulate what they do with their own cells.

Stem cells, found in both embryos and adult tissues, offer enormous promise to scientists because they have the potential to develop into many different kinds of cells or serve as the body's own repair service.

Research is exploding into ways stem cells might be harnessed to cure diseases, mend damaged tissue, even grow replacement organs.

But most such research is still in the early stages. To date, the FDA has approved only a handful of stem-cell treatments, mainly for blood diseases such as leukemia. Scientists say much more work needs to be done to understand how stem cells work and what uses are safe and effective.

"We need to make sure that these technologies are reliable and reproducible, time and time again, before you put them into patients," says Anthony Atala, direc-



SHAWN ROCKAFELLOW; BRIAN DIKMAN AND JOHANNAH SANCHEZ-ADAMS



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Shawn Rockafellow was going blind and had stem-cell therapy at a clinic. His regular ophthalmologist, Dr. Scott Markham, right, with friends at his 90-day checkup, agrees that Mr. Rockafellow has improved, but can't explain why. Stem cells, above.

Stem Cells in the Lab | Where scientific stem-cell research stands on some common conditions

- ◆ **Arthritis** Researchers are using stem cells to reduce inflammation, repair bone and cartilage damage and create artificial hip tissue. Tests in humans are several years away.
- ◆ **Diabetes** Scientists are working to reprogram adult stem cells from skin to become functioning beta and immune cells, which malfunction in diabetes.
- ◆ **Heart disease** Clinical trials in Europe are testing whether bone marrow stem cells infused into coronary arteries can repair damage from heart attacks. Researchers have also created beating heart tissue from stem cells in a lab that might be used to patch damaged hearts someday.
- ◆ **Macular degeneration** Researchers are using reprogrammed stem cells from skin to grow new rods and cones. But getting them to connect with nerve fibers is challenging.

tor of the Wake Forest Institute for Regenerative Medicine in Winston-Salem, N.C., which has 450 researchers working to create new tissues from stem cells.

"This is the future of health care, using your own stem cells to fix problems, not drugs," says Paula Grisanti, chairwoman of the National Stem Cell Foundation, a nonprofit that funds many research projects.

"But clinics that make over-the-top claims that a single stem-cell therapy will cure ALS or Parkinson's or other diseases raise huge safety and ethical concerns. It gives the whole field a black eye."

Some clinic operators say they are offering "patient-funded research" and that the charges are modest if the treatments restore sight or forestall a knee replacement.

Jeffrey Weiss, a retinal surgeon in Margate, Fla., has treated about 570 patients with retinal and optic nerve diseases with stem cells taken from patients' bone marrow as part of a study, and says that about 60% have had meaningful improvement. Patients pay \$19,000 to \$21,000 to receive the injections.

Shawn Rockafellow, a 31-year-old truck dispatcher in Chandler, Ariz., started rapidly losing his vision in 2014 to a genetic disease and says he was told to accept that he was going blind. His mother read about Dr. Weiss's work.

Mr. Rockafellow raised the \$20,000 fee on GoFundMe, a personal charity website, and had the treatment in both eyes in January. After three months, the vision in his right eye went from roughly

and if they perform their original role in the target location.

Some scientists dispute that reasoning. "Fat stem cells come from fat, which has almost no role beyond cushioning," says Paul Knoepfler, a stem-cell researcher at the University of California, Davis who co-wrote the Cell Stem Cell study.

Mark Berman, a Beverly Hills, Calif., cosmetic surgeon who co-founded a network of stem-cell clinics, says "fundamentally, all we are doing is a simple, surgical procedure. This is not witch-doctor stuff. We are repairing cell damage with people's own stem cells." He says the member clinics in 25 states have treated about 5,000 patients to date, with no significant adverse events.

SammyJo Wilkinson, a former dot-com executive, developed multiple sclerosis in 1995 and was confined to a wheelchair by 2011. She says her symptoms started to improve almost immediately after receiving a high-dose stem cell treatment at a Houston clinic in 2012.

When the FDA blocked access to that form of therapy, Ms. Wilkinson went to Cancun, Mexico, for follow-ups. After a total of five treatments for \$90,000, she says she has far less pain, can exercise and walk short distances with the help of a walker.

At the FDA hearing, Ms. Wilkinson, who founded a patient group called Patients for Stem Cells, plans to appeal for a faster approval process for stem-cell therapies and a registry to monitor patient outcomes.

"Patients will never get these treatments if they have to go the traditional double-blind placebo-controlled trial route. That takes 10 years and \$1 billion," she says. "There's got to be a middle ground, where you don't shut off treatment, you just keep track of it."

Some researchers say the process shouldn't be hurried too much.

"I understand that patients for whom there are no other good options want access now," Dr. Grisanti says. "The hard, sad truth is there has to be oversight to make sure what you are getting is going to benefit and not harm you."

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20/1,000 to 20/400. After six months, it was 20/300. His left eye hasn't improved as much, so he wants to try the treatment again. His regular ophthalmologist, Scott Markham, says "the fact that he's not worsening is fantastic."

Many clinics use so-called adult mesenchymal stem cells derived from fat. In a mini liposuction procedure, doctors withdraw a syringe full of fat from the patient's abdomen under local anesthesia, inject them back into the patient, where they naturally seek out and find damaged tissue, proponents say.

Clinic operators say they don't need FDA approval because under the agency's draft guidelines, stem cells aren't considered drugs if they are the recipient's own cells, they are not significantly altered