

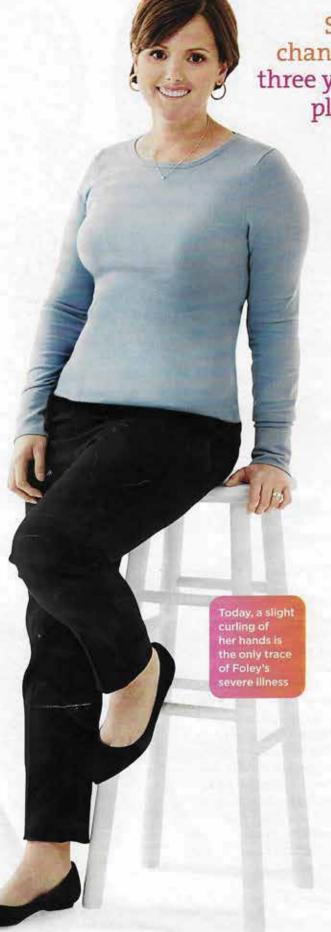
SPECIAL REPORT

Could a Petri Dish

The new science behind stem cells is treating incurable diseases. But there's also fraud and false hope around this miracle medicine. Here's what to know

BY AMANDA ROBB PHOTOGRAPHS BY DANA GALLAGHER





She was given less than a 50% chance of surviving five years. "So three years after my wedding, I was planning my funeral" –Molly Foley

The rheumatologist took one look at Foley's skin and said he didn't think it was arthritis. He thought what she had was worse—much worse. Blood work, a skin biopsy, a CT scan of Foley's chest, and an echocardiogram of her heart confirmed his diagnosis. "I had systemic scleroderma," Foley says.

Scleroderma is a chronic connective tissue disease. It occurs when the body turns on itself, destroying the smallest blood vessels and smooth muscle cells and replacing them with collagen and scar tissue. The collagen builds up, and affected organs harden. Scleroderma has no known cause and no conventional cure. Without treatment, Foley was given less than a 50% chance of surviving five years. And even with treatment—aggressive immunosuppressants—more than a third of patients die anyway, she learned. "So three years after my wedding, I started planning my funeral," Foley says.

But as she pecked out mourning instructions that she hoped would help her loved ones—a service at the church where she and her husband had been married, a big "happy" wake, bagpipes playing "My Wild Irish Rose"—Foley made appointments with two doctors working along what appeared to be a fine line between science and science fiction. "They were experimental stem cell studies, where the doctors were 'reprogramming' the immune system," Foley says. "Of course that sounded great. And of course I wanted hope. But I felt like I had to be very, very careful. Because I really wasn't interested in false hope—for me or my family."

REAL SCIENCE, REAL HELP

oth doctors Foley consulted were well-credentialed and involved in research at universities with rigorous oversight. She discussed the studies with her physicians and her family, and she got info from staff at the Scleroderma Foundation, a group she had contacted shortly after her diagnosis. Then she looked up the medical literature on both, checking that the work had been published in peer-reviewed journals (meaning it had been vetted by other doctors). "I'm just a super-thorough person," Foley says. "I can't help it."

After her considerable research, which included making sure that she met all the criteria to participate.

Foley chose to enroll in a study conducted by Richard K. Burt, M.D., chief of the Feinberg School of Medicine's division of immunotherapy at Northwestern University in Chicago. The main reason Foley picked it: Patients in the control group (who did not get the new treatment) were able to "cross over" and receive a stem cell transplant if after one year the disease progressed. Since assignments are always done anonymously and randomly, "there was a 50% chance of winding up in the control group," Foley says. "And I didn't see the point of signing up to help test an experimental treatment if I wasn't assured of the chance to at least try it."

Dr. Burt agrees that all patients should have that option. Right now, he explains, the standard is that you're given a drug, and then you have a life of managing your illness. "But stem cell treatment is a paradigm shift in medicine. You treat one time, then use medication, and eventually stop all therapy unless the disease comes back."

Sure enough, Foley wound up in the control group. For six months, she received high doses of a conventional treatment for scleroderma, the immunosuppressant Cytoxan. She did not get any better. Instead, she got worse. She couldn't walk more than a few feet without stopping. She couldn't bend to put on her own socks. Her lung function dropped below 50%, and she was facing heart trouble.

Finally, in March 2009, Foley got to try stem cell therapy. "I couldn't believe how simple it was," she says. First, in a procedure that took two and a half hours, doctors used a dialysis-type machine to extract stem cells from Foley's blood. Then, two weeks later, she returned to the hospital and underwent five days of high-dose immune suppressant therapy to completely knock out her malfunctioning immune system. A week after that, doctors brought Foley's stem cells back to her room—the same cells, not treated in any way. She asked a hospital minister to bless what looked to be a pint-size bag of blood. As soon as the prayer was over, a nurse hooked Foley up for an ordinary transfusion. The whole procedure took about 20 minutes.

"I started feeling better immediately," Foley says. "An occupational therapist came to work with me during the 10 days I had to stay in the hospital. Right away, we both noticed that the skin on my hands loosened up. I could move my fingers. I could stand and sit without joint pain. When I got out of the hospital, I started physical therapy. By June, I could ride a stationary bike for 10 minutes. By July, I could get my hands over my head. And late that summer, I got out my golf clubs and started chipping balls in my backyard."

Treatments like Foley's are at the cutting edge of medicine, taking these most continued on page 190



Stem Cells Are Helping...

HEART DISEASE

Doctors at Cedars-Sinal Heart Institute in Los Angeles and Johns Hopkins University in Baltimore have successfully used cardiac stem cells to regrow damaged heart muscle after a heart attack.

DIABETES

Using patients' own immune cells as well as stem cells from cord blood, University of Illinois at Chicago researchers have lowered blood glucose levels in 15 type 1 diabetics for 40 weeks.

MACULAR DEGENERATION

Doctors at UCLA modified embryonic stem cells and improved the vision of two women with macular degeneration. One can now thread a needle; the other can navigate a shopping mall.

CEREBRAL PALSY

Joanne Kurtzberg, M.D., of Duke University, has just begun a study using stem cells derived from cord blood to treat children with cerebral palsy. Earlier research suggests this can help, but "we can only know for sure with a controlled trial," she says.

OBSTRUCTED VEINS

Last spring, Swedish doctors saved a 10-year-old girl's life by inserting a bioengineered intestine-to-liver vein in her body to replace her obstructed one. They seeded a cadaver vein with the child's stem cells, creating a part that will grow as she does.

SPINAL CORD INJURIES

The first human embryonic stem cell clinical trial for paralysis was stopped last year (with the company citing financial reasons). But in July, UC Irvine received a \$20



Stem Cells

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basic biological units—essentially, cells that don't yet have a specific identity (muscle, skin, hair)—and using them to create a healthy immune system or, in other cases, to repair diseased tissue. But patients have actually been getting stem cell treatments for more than 40 years in the form of bone marrow transplants, bone marrow cells being the only kind doctors knew how to retrieve and eas-

ily transplant for many years. While the approach became highly successful, it was limited to treating a few cancers and blood disorders. Starting in the late 1980s and 1990s, however, scientists developed tech-

niques to collect stem cells from a variety of sources (including newborns' cord blood) as well as to modify an individual's own stem cells and grow them into new tissue; over the past decade, doctors have been trying out this technology on a host of different diseases and conditions, from diabetes to Parkinson's disease. Today there are nearly 1,800 FDA-approved stem cell clinical trials being conducted in the United States.

But as research expanded, so did the controversy. Some stem cells are obtained from human embryos created for in vitro fertilization use; the embryos are destroyed in the process of harvesting the stem cells. In 2001, to quell the controversy, President George W. Bush offered a compromise, restricting federal funding for research to already-existing lines of stem cells. But many scientists found that too restrictive and sought private funding to continue their work. Then, in 2009, President Barack Obama lifted the restriction.

So far, only a handful of clinical trials have used embryonic stem cells, though many scientists believe they're more promising than those obtained from other sources because they're



"The ophthalmologist said there was no improvement. But now my 'blind' daughter has just passed her driver's test"—Rochelle Morse

the most adaptive, able to grow into virtually any type of body cell—nerve, heart, cartilage—explains Doug Melton, Ph.D., codirector of the Harvard Stem Cell Institute. Others are less flexible: Liver stem cells, for example, can grow only into liver cells.

There's also controversy about some practitioners of stem cell medicine. Beyond the legitimate doctors testing experimental treatments, there are many charlatans who are offering nothing more than false hope—and possibly danger. It can be extraordinarily difficult to discern the hope from the hype, and desperate patients and their families often wind up making life-altering medical decisions—and spending huge amounts of money—based only on Google searches and misplaced faith.

A "MIRACLE" IN CHINA?

ochelle Morse first learned about stem cells seven years ago. She was doing her quarterly Internet search—"Optic Nerve Hypoplasia" and "Treatment"—when she saw a story about frogs' ability to regenerate optic nerves. Oh, wow, Morse thought. Maybe someday stem cells will help my daughter!

Morse's older child, Macie, was born with optic nerve hypoplasia (ONH), a condition in which the optic nerve—which transmits visual information from the retina to the brain—is underdeveloped. Macie was diagnosed in 1993, when she was 2 months old. "The ophthalmologist said, 'She is blind. She will always be blind. It is what it is, and there is nothing you can do.' I remember it ⇒