New stem-cell treatment: 'Hype is ahead of the science'

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Before New York Yankees pitcher Bartolo Colon pulled his hamstring while running from the mound to first base on June 11, fans would have been forgiven for thinking he had chugged from the Fountain of Youth.

Colon has not completed a full season since 2005 and sat out 2010 to rest his aging and injured right arm. But this season, his fastball is back. His ERA, 3.10, was among the tops in the league. On May 30, six days after his 39th birthday, he pitched his first shutout in five years, hurling his final pitch at 95 mph.

What lit the fuse on his fastball? An infusion of stem cells, says Joseph Purita, founder of the Institute of Regenerative and Molecular Orthopedics in Boca Raton, Fla., who gave Colon the controversial treatment in the Dominican Republic before baseball season began.

Purita isn't the only doctor offering patients stem cells. Doctors in the U.S. and abroad are now providing untested and unapproved stem cell therapies for ailments ranging from heart disease to emphysema to cerebral palsy. And they swear by them.

"Here's a guy who was fooling around for two years and not getting any better. All of a sudden, you do this procedure and a few weeks later he's dramatically better," Purita says. "There must be something going on here."

Experts liken stem cells to the seeds from which many body tissues

grow. If scientists can harness stem cells in healing, researchers say, they can revolutionize medicine. Embryonic stem cells-those derived from human embryos-hold special appeal because they can give rise to every cell type in the human body. More recently, researchers have raised the possibility that "induced" stem cells created from skin cells may have the same potential. As a result, world-class scientists agree that stem cell therapies may someday help to rebuild failing hearts, restore cancer-ravaged tissues, bridge gaps in nerves or regenerate damaged lung tissue.

"We really could repair faulty or damaged tissues," says George Daley, director of stem cell transplantation at Children's Hospital Boston, whose team infuses stem cells into leukemia patients as a component of bone marrow transplants. These transplants, which involve destroying cancerous bone marrow and replacing it with disease-free stem cells, have been the standard of care for leukemia for decades.

Daley says bone marrow transplantation remains the only proven form of stem cell therapy. "Virtually everything else is highly experimental," he says, including the most controversial stem cells of all, those derived from human embryos.

It may seem that Colon's treatment turned back the clock, experts say, but there's no evidence that the infusion had anything to do with it.

"We have no way of knowing even whether stem cells are the active ingredient," Daley says.

Even Purita acknowledges that he can't be sure Colon's improvement resulted from his therapy. "Can I be 100 percent sure it was the stem cells? No, I can't be sure," says Purita. MLB is investigating to make sure Colon wasn't given any banned substances. Purita says he would never take that risk. He also says he limits his stem cell practice to his orthopedics specialty. "I'm not trying to treat Alzheimer's and strokes."

Yankees spokesman Jason Zillo says Colon declined to comment.

Ted Kaptchuk of Harvard Medical School says he's not surprised that some patients appear to benefit from stem cell treatments. That's not necessarily because the treatments work, he says. What matters is that patients think they work.

Kaptchuck says medical history is filled with studies in which sugar pills and sham surgery out-perform the real thing, a phenomenon called the "placebo effect." The placebo effect is especially potent in surgery, he says, noting more than 100 studies in which "people do wonderfully on the placebo." In one powerful example, he says, a researcher tracked patients for two years after half had real surgery and half had a sham procedure for arthritis of the knee.

The patients who had fake surgery, Kaptchuck says, "were hopping around, doing great. There was no difference between the sham surgery and the real surgery." He adds: "When you go under the knife, it's like going to a shaman. The only difference is that there are no feathers, there are machines and test tubes."

An Internet search for "stem cells" will turn up a roster of doctors who offer purported stem cell treatments. Most use adult cells from the patients themselves. No one knows how safe or effective the procedures are, researchers say, because few, if any, of the doctors now offering them to patients have tested them scientifically.

"It's a case where the hype is ahead of the science," says Gary Green of the University of California, Los Angeles, medical director of Major League Baseball.

Even doctors working on FDA-approved stem cell clinical trials are still figuring out how to formulate treatments, deliver them effectively and

achieve maximum potency. Reliable therapy is years away. When patients agree to undergo unapproved therapies, they are taking a leap of faith, based on little more than the word of their doctors and the encouragement of other patients.

"We'd all love easy miracles," says Larry Goldstein, head of stem cell research at the University of California-San Diego. "That's not the way it works."

In most cases, stem cell doctors extract the cells directly from the patient's blood, fatty tissue or bone marrow. They use standard laboratory methods to separate them from the blood or other substances. Doctors at some offshore clinics may also obtain stem cells from umbilical cord blood or, in rare cases, human embryos. Doctors then inject or infuse the slurry of concentrated cells back into the patient, where, both doctor and patient hope they'll promote healing. These approaches-especially the use of human embryonic stem cells-have not been approved by the Food and Drug Administration.

The most coveted stem cells are those from embryos, which can morph into any cell in the human body. The Bush administration, to discourage embryo destruction, put strict controls on embryonic stem cell use.

The government would only fund research using certain batches of stem cells that had already been harvested from embryos. The Obama administration lifted those restrictions, expanding federal funding of embryonic stem cell research. The move was temporarily blocked by a judge, but on April 29, the U.S. Court of Appeals for the D.C. Circuit overturned the ruling and threw open the doors to more federal funding. Those restrictions only applied to research funded by the government, not studies carried out by privately funded biotech firms. Most studies, public and private, are just getting underway.

FDA regulations have loopholes, Goldstein says. FDA guidelines limit its authority to regulate treatments involving cells that are withdrawn from a patient and then infused the same day with only "minimal manipulation." Last August, in a test of its authority, the FDA requested an injunction from the U.S. District Court in Washington, D.C., to block a Broomfield, Colo., orthopedic clinic, Regenerative Sciences, from formulating treatments of cultured stem cells.

The clinic's medical director, Christopher Centeno, says he has repeatedly sued the FDA, arguing that these treatments fall within FDA guidelines for the practice of medicine. The FDA countered with its own lawsuit. The dispute won't be decided until 2013, Centeno says. The FDA declined to comment because the case is pending.

Centeno says he is trying to move stem cell therapy into the mainstream. He helped establish the International Cellular Medicine Society, which drafted guidelines for stem cell treatments and promotes responsible research, he says.

The group does not yet accept members, but about 1,500 people, half of them doctors and half patients, have signed up for information, says David Audley, the group's executive director. Two clinics in the U.S. are going through the group's stem cell accreditation process. The group's goal, Audley says, is to assure patient safety, by tracking patients for up to 20 years through a patient registry. "At this point, we don't have enough data to talk about true efficacy," he says. "We would love to get into efficacy."

Buyer beware, warns Daley, who is also the head of the International Society for Stem Cell Research, an academic group that posts a cautionary handbook for consumers on its website.

"On one hand," he says, "there are charlatans selling snake oil. At the

other end of the spectrum are physicians who may be well intentioned, but they're misinformed if they're giving patients stem cells before they've been proven to work."

Many patients say they can't, or won't, wait years for scientists to gather evidence, as long as there are doctors willing to treat them now.

Barbara Hanson, founder of the online discussion forum Stem Cell Pioneers, says stem cells have allowed her to rebound from life-threatening pulmonary disease and resume an essentially normal life.

"We're not going to sit here and just die, and wait for the FDA to give its stamp of approval for us to have our stem cells used," she says.

Clinics are flourishing in the United States, Mexico, China, India, the Dominican Republic, Thailand, Russia and the United Kingdom, says Tim Caulfield, a University of Alberta, Canada, law professor who has studied "direct-to-consumer" Internet marketing of stem cells. Caulfield's team found that most websites play up the benefits and downplay the risks of stem cell therapy.

The average cost of care: just under $50,000. Nearly half of those treated were under 18, he says. Parents often fly children to clinics in other countries for untested stem cell treatments for such ailments as autism and cerebral palsy.

"It makes me angry," he says. "They're trading on the excitement of stem cells to market these therapies all over the world, for everything you can think of," including autism, multiple sclerosis, blindness, heart disease, cancer, neurological disorders, even aging. "I regard that as a marker for quackery. If they treat everything, you know it's too good to be true."

Increasingly, doctors find themselves treating critically ill patients who

sought stem cells first.

Cleveland Clinic heart specialist Wael Jaber says he was "astonished" when a 75-year-old patient told him he paid $50,000 for stem cell treatments to heal his ailing heart. Two Mayo Clinic lung experts, Charles Burger and Neal Patel, say a pair of their patients paid roughly the same amount for stem cells to treat a deadly lung disease.

"They paid cash, 50 to 60 grand, in advance," Burger says. Medical tests showed that none of the three benefited from the therapy, Jaber and Burger say.

Jaber says his patient-who had triple bypass surgery June 1 - sought his stem cells at The Brain Therapeutics Medical Clinic in Mission Viejo, Calif., run by osteopath David Steenblock, who advertises on a website called StemcellMD.org.

Steenblock says he treats patients with heart disease, diabetes, stroke, seizures, Parkinson's disease, cerebral palsy, Lou Gehrig's disease , or ALS, kidney failure and chronic lung disease. He refers some patients to a clinic in Mexico.

Steenblock's record is anything but unblemished. California Osteopathic Medical Board records show that the board revoked Steenblock's license in 2009 for gross negligence, excessive prescribing and dishonesty while treating a stroke patient with hyperbaric oxygen, which is also controversial. The revocation was stayed; Steenblock may continue to practice medicine, but he was placed on five years' probation, during which he was required to take a course in medical ethics. Steenblock acknowledged the judgment against him, but said his license is "unfettered" and he's appealing the ruling.

Steven Nissen, chief of cardiology at the Cleveland Clinic, was so

incensed when Jaber told him about his stem cell patient that he fired off a letter of complaint to the FDA. Jaber says he was interviewed by FDA investigators.

The two Mayo clinic patients told their doctors they had their blood drawn at the Regenocyte Therapeutic clinic in Bonita Springs, Fla., run by Zannos Grekos. Grekos says he sent the blood to Israel for processing. It was then shipped to a hospital in Santo Domingo, the Dominican Republic, where the cells were infused into the patients.

Grekos also has had his problems with state regulators. On Feb. 23, the Florida Board of Medical Examiners filed an emergency order blocking Grekos from providing stem cell therapy in Florida. The state took action after Grekos infused bone marrow cells into the "cerebral circulation" of a 69-year-old woman suffering from a stroke.

The woman, who got the infusion as an outpatient, fell at home that evening and later died of injuries from her fall, records show. A Florida prosecutor, Robert Milne, charged that the treatment was "entirely experimental" and had no "substantiated medical and/or scientific value."

"We're going to request a hearing and present our side," Grekos says. He notes that while he can't do stem cell procedures in Florida, he's still doing the infusions in other places, including the Dominican Republic and Athens.

Steenblock and Grekos reject the criticism of their efforts. "Some (doctors) are more conservative, some are more progressive," Grekos says. "If we didn't have more progressive physicians, medicine wouldn't move forward."

Nevertheless, says Mayo's Burger, "This isn't an approved study or a

therapy approved for use in the U.S."

The challenge, he says, is to find a balance between protecting patients and allowing doctors enough latitude to innovate and carry out legitimate research. "You don't want to slam the door on something that in 10 years may have potential for people with end-stage disease," Burger says.