

The other stem cells

Although embryonic stem cells dominate public discussion, a number of companies are building treatments, and businesses, around adult stem cells

By Diedra Henderson, Globe Staff | June 20, 2005

BALTIMORE -- As Congress spent hours passionately debating the merits and ethics of embryonic stem cell research, nearly 50 Osiris Therapeutics Inc. employees here continued their quiet work on less controversial adult stem cells.

Thanks to Food and Drug Administration fast-track designation, Osiris expects to have an adult stem cell-based therapy on the market by late 2007 to combat potentially fatal tissue rejection among leukemia patients undergoing bone marrow transplants.

That's one of three adult stem cell-based therapies Osiris currently has in human trials, to the delight of investors who muscled their way into the company's latest funding round to raise \$50 million, more than double the expected amount.

Another human clinical trial squirts adult stem cells into damaged knees after surgery to regrow meniscus, restoring the tissue that acts as a shock absorber and preventing onset of arthritis. The third experimental therapy being tested in humans -- including patients here at the Johns Hopkins University School of Medicine -- uses adult stem cells to help replace tissue damaged by heart attacks.

Meanwhile, a University of Pittsburgh researcher is tapping adult stem cells in an FDA-approved, university-financed safety trial to rally these cellular repairmen to help fix failing hearts.

"When you inject these cells in, they act like a homing beacon to the heart," said Dr. Amit Patel, director of the university medical center's cardiac cell therapy center. "The heart's just sending out an SOS signal saying 'Here! Come help me,' " Patel said. The adult stem cells then enlist other cells that deliver building blocks needed to partially restore heart function. Because the patients in the trial are awaiting heart transplants, Patel and others will be able to study their original heart after transplant to determine the impact of stem cell therapy.

To Wall Street, stem cells are more than simply the latest issue on which conservative members of Congress disagree with their more moderate counterparts. Stem cell therapies, within a decade, stand a chance of producing earnings for investors.

Embryonic stem cells are blank-slate cells that, in petri dishes, transform into most any type of cell. While embryonic stem cells possess more therapeutic potential, research remains controversial -- and minimally funded by the federal government -- because current techniques destroy human embryos as stem cells are extracted.

Adult stem cells, derived from such noncontroversial sources as umbilical cord blood and bone marrow, transform into fewer types of cells. But because adult stem cells sidestep the ethical issues that surround embryonic stem cells, therapies based on adult stem cell may have a speedier path to the marketplace.

The progress that the human clinical trials using adult stem cells represents is "unbelievably significant," said Dr. Alan Levine, former director of the blood disease program of the National Heart, Lung and Blood Institute at the National Institutes of Health.

If the trials result in therapies that earn FDA approval, "that would change the whole complexion of the way certain diseases are treated," Levine said. "If someone has a massive heart attack and a good portion of their heart muscle dies, it doesn't come back to life."

Dr. Joshua Hare, principal investigator of the phase I safety trial for Osiris' heart repair therapy, said stem cell insights now have emboldened researchers to view repair of damaged organs, like the heart, as possible.

"We think this is one of the most exciting new innovations in heart attack therapy," said Hare, a Johns Hopkins cardiologist. "Every 10 or 20 years, there is a brand-new insight. I would say that what is happening with stem cell therapy for these chronic diseases is sort of the new insight for the 21st century."

Many orthopedic surgeons maintain a "healthy skepticism" because creating replacement cartilage as durable as the original material remains complicated.

"Clinically, we realize that there's a long way from a phase I all the way to a phase III study -- and then getting FDA approval," said Dr. Jay Lieberman, an orthopedic surgery professor at the David Geffen School of Medicine at the University of California, Los Angeles. "I think the orthopedic community is anxiously awaiting a therapy that would be an improvement on the techniques that we're using today. However, my guess is that would be a minimum of five, perhaps even as long as 10 years away."

Just one year ago, Osiris faced skepticism in the market and had been hit with a multimillion-dollar wrongful termination suit filed by a former CEO. That suit was settled, said C. Randal Mills, the company's current CEO. Mills arrived last July with a mission to sharpen the company's focus on bringing products to market.

Osiris expected to raise \$20 million in its latest funding round but now finds itself with enough funds to carry the company through 2006.

"It allows us to have three clinical trials that are active and aggressively moving forward. Clinical trials are very, very expensive propositions," Mills said. "Not only are we going to start these trials, but we have the financial means to finish these trials, as well. We have the resources to actually bring these products to market."

One of the more sobering safety issues raised by therapeutics harnessing adult stem cells is the possibility the cells could form tumors, which was suggested by a paper that appeared in the April 15 issue of *Cancer Research*, an American Association for Cancer Research journal.

Mills said the company uses a technique that expands a single donation into enough stem cells for 15,000 treatment doses. That's a fraction of the potential yield, but stops the expansion process well before the phase when the cells could pose tumor risks.

"We keep the cells in a more healthy state and we just flat out avoid all of the risk that comes along from having the cells become cancerous," he said. "What we're really interested in doing is making sure we have the highest quality, most effective drug product we can have. We don't want to compromise that for an increase in yield. We'll bring in more bone marrow and make more cells from a different donor."

While Osiris' tissue-rejection product could take the speediest path to patients, the potential life-saving therapy would serve a niche market. Other therapies, however, could help hundreds of thousands of aging baby boomers with weakened knees and seniors with failing hearts.

Roughly 800,000 Americans undergo meniscus surgery annually, while another 400,000 shun the procedure, fearing arthritis can follow, Mills said. Some 1 million Americans each year suffer their first heart attack, he said.

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