

April 8, 2008

Stem-Cell Studies in People Scrutinized by U.S. FDA on Safety

BY ELIZABETH LOPATTO

April 8 (Bloomberg) -- Embryonic stem cells may someday cure disease, reverse paralysis or restore memory. Scientists worry they also may trigger benign tumors, and even cancer.

[Geron Corp.](#) and [Advanced Cell Technology Inc.](#) plan this year to begin the first human tests of therapies created with stem cells extracted from human embryos. The U.S. Food and Drug Administration, which may soon approve the studies, is asking the companies to closely monitor whether the therapies can spur non-cancerous growths, as has been seen in animal tests.

The first human studies, by Menlo Park, California-based Geron, may start as soon as this summer in patients partly or wholly paralyzed. The FDA is convening a special public session April 10 to discuss the safety concerns. The agency scheduled the meeting to get help in balancing the pleas of patients with risks that may arise should the therapies gain wide use.

"The FDA officials are deer in the headlights, caught between the tensions of the public," said Carol Pratt, who specializes in FDA regulations as a partner in law firm Kirkpatrick & Lockhart Preston Gates Ellis LLP, in Portland, Oregon.

The FDA is under public pressure to release new treatments quickly, and it must also assuage safety concerns that have resulted when approved medicines were found to have dangerous side effects after they gained widespread use, Pratt said.

"Don't stand in the way of new medical therapies, and for God's sake, don't let any American get hurt," Pratt said of the mixed regulatory message the FDA hears. "They don't know where to draw the line on stem cells right now, and there's no way they would."

Geron fell two cents to \$4.87 in Nasdaq Stock Market composite trading yesterday. It dropped 34 percent in the 12 months before today. Advanced Cell rose 2 cents to 13 cents in over the counter trading. It fell 84 percent in the 12 months before today.

Criticism

The agency has faced U.S. congressional criticism of its oversight of new drugs after elevated heart risks prompted the withdrawal of [Merck & Co.](#)'s painkiller Vioxx in 2004. Last year, studies found [GlaxoSmithKline Plc's](#) diabetes pill Avandia raised heart risks as well.

The [Government Accountability Office](#), Congress's Washington-based investigative arm, said on March 4 that it will investigate whether the FDA approves drugs without enough evidence of safety and effectiveness. The stem-cell companies welcome heightened scrutiny, they said.

“You don't want a tooth growing in your eye, so we need to absolutely know what will happen,” said [Robert Lanza](#), the medical director for Alameda, California-based Advanced Cell, in a telephone interview. “It's not in anyone's interest to have any risk whatsoever.”

Eye Disease

Scientists discovered how to obtain stem cells from human embryos and cultivate them in the laboratory in 1998, according to the U.S. National Institutes of Health. Since then, researchers have begun coaxing the cells to grow into different tissue types that can help patients repair diseased organs.

Advanced Cell is testing therapies derived from embryonic cells for treating eye diseases, a market it estimates at \$28 billion. The company is aiming at curing diseases including [macular degeneration](#), an illness that weakens the part of the eye responsible for sharp vision and affects about 15 percent of people over the age of 75, according to the NIH.

[Novocell Inc.](#), a closely held company based in San Diego, is creating insulin-producing cells for diabetics. Geron is attempting to treat spinal cord injuries by turning stem cells into a type of nerve cell that insulates the spinal cord. At the FDA session this week, the three companies will discuss ways to monitor safety so clinical trials can proceed, they said.

\$3 Billion

“We're ready now to fund phase 1 trials,” said [Marie Csete](#), the chief scientific officer for the [California Institute for Regenerative Medicine](#), in a telephone interview. “It would be wonderful if we saw these trials proceeding as partners with the FDA.”

The San Francisco-based stem cell institute was established after California voters approved Proposition 71 in 2004. The measure authorized the state to sell \$3 billion in bonds to fund research on stem cells, especially those derived from embryos, over a 10-year period.

Early clinical trials with stem cells provide different challenges than typical drug trials, the FDA staff wrote in documents filed last week on its Web site.

Of particular concern to regulators are so-called [teratomas](#), non-cancerous tumors that can contain a mixture of different types of cells. Growth of such tumors in the spine or brain would be harmful, according to the FDA's documents. In studies with animals whose immune systems are suppressed, injected human embryonic stem cells create teratomas.

Guidelines

“It's hard for me to imagine a hard, fast guideline that would apply to all the diseases you could treat with stem cells,” said Melissa Carpenter, the vice-president of research and development at Novocell. “Our goal is to learn what the FDA is thinking, what other groups are doing, and what technologies might help us achieve safety.”

Geron declined to comment on the contents of the meeting, outside spokeswoman Tracey Milani of Russo Partners wrote in an e-mailed statement.

To contact the reporter on this story: [Elizabeth Lopatto](#) in New York at elopatto@bloomberg.net.

