

# Political factors remain as stem cell science advances

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Advanced Cell Technology Inc. is pioneering new science.

The biotech company, with facilities in Marlborough, is among one of the first U.S. companies to win FDA approval for human clinical trials for drug therapies using embryonic stem cells. In fact, in the past year the company has actually gained approval for two such treatments.

“What it demonstrates to the world is that the FDA is willing to work with companies trying to bring products made with embryonic stem cells to the market,” said Matthew Vincent, business development director at the Santa Monica, Calif.-headquartered company.

ACT is just one of a number of private companies and public institutions in Massachusetts moving forward with embryonic stem cell research. The pace of future successes, however, is unclear — even with a recent, major legal turning point, the issue of embryonic stem cells remains politically charged.

The U.S. Court of Appeals for the District of Columbia Circuit in April overturned a preliminary injunction that had banned federal spending on research involving human embryonic stem cells. Even so, the future of federal funding for such research remains uncertain.

Vincent said he expects the field of stem cell research to produce significant breakthroughs soon, despite such concerns and uncertainty.

“We’re right at that tipping point,” he said.

There’s evidence of that already. ACT’s two approvals, both granted near the end of 2010, follow the 2009 Food and Drug Administration’s approval of human studies for Menlo Park, Calif.-based Geron Corp.’s treatment for spinal cord injuries — the first-ever human trial of a medical treatment derived from embryonic stem cells.

ACT’s first drug to receive the FDA’s OK for clinical trial uses retinal cells derived from human embryonic stem cells to treat patients with Stargardt’s macular dystrophy, a common form of juvenile macular degeneration that eventually leads to blindness. There’s currently no treatment for it on the market.

Its second drug treats dry-age-related macular degeneration using retinal pigment epithelial cells derived from human embryonic stem cells. There are no treatments available for Dry AMD, the most common form of macular degeneration, affecting between 10 million and 15 million Americans.

Vincent said the FDA’s decisions to clear treatments using hESCs for trial are significant for the potential therapeutic benefits. But the FDA’s actions are also significant for its industry potential, signaling to private investors its financial potential.

That holds a unique importance in the field of stem cell research because of the political debate that has followed this area from the start. The controversy over the use of stem cells from embryos led Congress to restrict federal funding, which for decades has been the primary source of money for early-stage biological research of all kinds.

That federal restriction has a ripple effect through the scientific community, said B.D. Colen, a Harvard University spokesman.

“One of the biggest difficulties caused by the uncertainty of funding and the current legal environment is that bright young scientists trying to decide what to do with their careers may find themselves very nervous about going into stem cell research because they don’t know if this is something that will end up being funded in the normal way. And who wants to build their career on something that might not get funded?” he said.

Colen said most of the research on stem cells is still taking place at academic and medical institutions, although there is increasing interest from private pharmaceutical companies and biotech firms.

Harvard, not surprisingly, is invested in this area of work. The Harvard Stem Cell Institute is a collaboration of scientists at the university and its affiliates, including Beth Israel Deaconess Medical Center, Children’s Hospital, Massachusetts General Hospital and the Schepens Eye Research Institute. The largest collaboration of its kind, the institute is an umbrella organization of more than 80 lab heads and 800 researchers, including graduate and post-doctoral fellows.

Of course, Harvard is not the only area academic institution supporting stem cell research. Other institutional research centers include MIT’s Whitehead Institute for Biomedical Research, BU’s Center for Regenerative Medicine and the UMass Medical School.

Still, the future of federal funding weighs heavily on the field, said Craig R. Smith, a

partner at Lando & Anastasi LLP.

He said researchers are encouraged by the Court of Appeals’ April decision overturning the preliminary injunction on federal spending. However, the injunction is part of a larger case still pending in lower court. That case questions whether the Obama administration’s decision to allow federal funding for stem cell research is allowed under the Dickey-Wicker Amendment, which prohibits the Department of Health and Human Services from using funds for the creation of human embryos for research purposes or for research in which human embryos are destroyed.

“There’s definitely concern in the industry because of the uncertainty on government funding,” Smith said.

Still, research continues in academic and medical institutions as well as private companies, supported with not just whatever federal funds they can get but also by nonprofits such as the Christopher and Dana Reeve Foundation and the Michael J. Fox Foundation as well as private investors and public shareholders.

“It’s kind of been a slow pace but there’s usually an inflection point where things just take off, and I think we’re getting to that point,” said Frank Reynolds, CEO, CFO and chairman of InVivo Therapeutics Corp., which has a product that in testing enabled paralyzed monkeys to walk again.

InVivo, a Cambridge-based biomaterials company, is working on producing therapies based on human embryonic stem cells; Reynolds said he expects the company to seek FDA approval for testing on these starting in 2013.

He said these therapies hold the most promise for treating spinal cord injuries and other injuries to the nervous system. And if more government funding comes available, Reynolds said, such promise could be fulfilled more quickly.



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A scientist at Advanced Cell Technology, one of the first companies approved for stem cell-related trials.