
From: Steven Victor MD [REDACTED]
Sent: 11/23/2012 1:06:38 PM
To: [REDACTED]
CC: [REDACTED]
Subject: Report on Stem Cells future

PUBLISHED: 22 NOVEMBER 2012

The view from the US: Stem cell therapy steps up a gear with first approval and improved political climate

Special Report

Peter Winter

Positive clinical data, increases in federal funding and the first regulatory approval of a manufactured stem cell product mean momentum is building in the US. With the re-election of President Obama, this is likely to continue

[Share on twitter](#)[Share on linkedin](#)[Share on facebook](#)[Share on email](#)



Image: Bigstock

When US President Barack Obama came into office he made good on his promise to overturn President George W. Bush's executive order that - with the exception of a handful of existing stem cell lines - prohibited federal funding of human embryonic stem cell (hESC) research.

Special Report - Stem Cell Research under Horizon 2020

[Stem Cell Research in Horizon 2020: Experts debate what Europe must do to maintain its lead in regenerative medicine](#)

[The view from the US: Stem cell therapy steps up a gear with first approval and improved political climate](#)

[View from Europe: Delivering on the vision of regenerative medicine and stem cells](#)

[Talking to the experts: Why the EU should support human embryonic stem cell research in Horizon 2020](#)

[OVERVIEW: Regenerate the future - human embryonic stem cell research is crucial to deliver health and growth](#)

This easing of restrictions on hESC research was expected to engender enough confidence to attract investors into the space and encourage pharmaceutical and biotech companies to build robust product pipelines based on stem cell therapies. However, a tougher regulatory climate for biopharmaceuticals in general and a protracted legal challenge to the relaxing of rules on hESCs has served to keep both big pharma companies and venture capitalists on the sidelines to date.

Nevertheless, there has been progress, particularly at the research end of the development spectrum. Three and a half years on stem cells are no longer high on the political agenda as they once were. This speaks to the generally positive public acceptance of stem cell research in the US. [A Research!America poll](#) of likely voters in the presidential election, conducted in August 2012, found that 61 per cent of prospective voters were in favor of expanding funding for hESC research.

Investor interest

Looking back over his first term in office Obama can point to some major gains. These include funding increases for the National Institutes of Health's (NIH) stem cell initiatives, and greater investor interest in the wider field of regenerative medicine including the use of adult stem cells and induced pluripotent stem (iPS) cells.

However, it hasn't all been smooth sailing. Although after protracted forethought, the US Food and Drug Administration (FDA) gave the green light for the Californian biotech Geron to begin the very first human clinical trial of a human embryonic stem cell-derived therapy in January 2009, the trial has not been without its problems.

The Phase I trial, treating patients with acute spinal cord injury, was hit with an FDA clinical hold causing significant delays to its initiation in 2010. A year later, after 15 years' effort, the Geron washed its hands of the programme, blaming its withdrawal from the stem cell space on capital scarcity and uncertain economic conditions. The decision wiped out a leading player in hESC translation and commercialisation. However, the regenerative medicine sector has been able to recover and is beginning to blossom. (See Public Markets section below). The prime movers in the Geron trial are now attempting to revive it.

Research funding robust

Federal funding for all forms of stem cell research has increased over the past four years. However, the NIH funding component for hESC has been dogged by litigation for the past three years. In *Sherley v. Sebelius*, researchers James Sherley and Theresa Deisher, who worked with adult stem cells, claimed the NIH guidelines violated the Dickey-Wicker Amendment, which prohibits the use of federal funds for research in which human embryos are destroyed or discarded. This overhang was not finally removed until August 2012 when a three-judge panel from the US Court of Appeals for the District of Columbia Circuit unanimously upheld the NIH 2009 guidelines that permit funding of hESC research.

Through its Common Fund the NIH has established the Center for Regenerative Medicine (NIH CRM), to support this field, with the goal of accelerating the translation of stem cell-based clinical therapies.

State funding filling the void

With stem cell research in general not attracting a significant amount of venture funding, the California Institute of Regenerative Medicine (CIRM), which was established in 2004 with \$3 billion for stem cell research at California universities and research institutions, has begun to fill the void left by traditional venture capital firms. To date CIRM has allocated \$150 million in funding to help move promising stem cell-based therapies from the bench into clinical trials.

"We are a lot closer to having promising therapies ready for clinical trials, so it makes sense that we step up our engagement with industry to help fund those trials and move those therapies closer to approval by the FDA," said Duane Roth, vice chair of the governing board of CIRM.

CIRM's funding for translational research is good news for biotech companies, providing them with a source of funds in a field where it remains challenging to raise private capital.

Three biotech companies have been funded so far under CIRM's Strategic Partnership Awards initiative. A grant of \$10.1 million was awarded to ViaCyte Inc. to continue preclinical research and initiate clinical testing of an embryonic stem cell-based therapy for patients with insulin-dependent diabetes.

Meanwhile, Bluebird Bio Inc. will use a \$9.3 million grant to support a Phase I/II study to evaluate the safety and efficacy of LentiGlobin, the company's programme for the treatment of the inherited blood disorder beta-thalassemia, which will be initiated in the US in 2013. StemCells Inc. has been awarded up to \$20 million to fund preclinical development of its product consisting of purified human neural stem cells for treating Alzheimer's disease, with the goal of filing for permission to carry out a clinical trial in that time. In July, CIRM approved a separate award to the company for up to \$20 million to fund preclinical development of a cell therapy for spinal cord injury.

Public Markets

It has been a good year for stem cell companies on the public markets, with the thirteen publicly-listed stem cell companies showing an average increase in share price of 11.6 percent in the year-to-date.

This boost came from a regulatory approval and positive clinical trials results. It was a big breakthrough for the field as a whole when the Canadian regulator Health Canada approved Prochymal, Osiris Therapeutics Inc.'s allogeneic stem cell treatment for graft-vs.-host disease (GvHD) in children.

The decision marked the world's first regulatory approval of a manufactured stem cell product and the first therapy approved for GvHD – a devastating complication of bone marrow transplantation that kills up to 80 percent of children affected, many within just weeks of diagnosis. The company's stock value has almost doubled in the course of this year.

Meanwhile, Newark, California-based StemCells, Inc. has seen its shares rise 148 per cent in the year to date. In addition to its CIRM grants, the company recently reported clinical and preclinical data demonstrating the therapeutic potential of a cell therapy for treating myelination disorders.

Pluristem Therapeutics Inc.'s stock value also has jumped 44 per cent, on the strength of reporting a single case study in which a patient with aplastic bone marrow who received an intramuscular injection of its PLacental eXpanded cells under compassionate use saw an improvement. The company was also able to successfully complete a public offering which netted about US\$30 million.

Positive clinical trials will begin to encourage investment

While still in their early stages of development - and with clinical trials having only involved a limited number of patients - reports to date have been very promising, and provide further validation for encouraging investment in stem cell therapeutics. For example, data from a human embryonic stem cell trial conducted by Advanced Cell Technology and published in medical journal The Lancet, showed that two patients with Stargardt's disease, a degenerative eye condition, had regained some vision.

In addition, positive early data from a spinal cord injury trial involving StemCells' neural stem cells indicated that two patients with no feeling below the site of injury were able to regain sensation, while in another study from the company, patients with a rare myelination disorder were able to create myelin, an advance that holds promise for treating multiple sclerosis and cerebral palsy.

This scientific progress has helped breathe a new sense of optimism into the US stem cell sector. The Fiscal Cliff apart, it seems likely this momentum will continue now Obama has secured a second term.

Peter Winter is a writer, editor and analyst on the global biotechnology industry. He is currently editor of BioWorld Insight.

Steven Victor MD
IntelliCell BioSciences
Chairman/CEO
460 Park Avenue 17th Floor
New York, New York 10022



www.IntelliCellBioSciences.com

