



“As to diseases ,make a habit of two things —
to help, or at least *do no harm.*”
— Hippocrates, *The Epidemics* —

Q & A

FDA APPROVES FIRST CLINICAL TRIAL OF HUMAN EMBRYONIC STEM CELLS

Q: Have any patients been treated with embryonic stem cells?

A: NO. To date, there are still no human patients who have been treated with embryonic stem cells.

- In contrast, adult stem cells (including stem cells from cord blood) have provided therapeutic benefits to human patients for 73 diseases and conditions.
- Most recently, adult stem cells were used in a trial that reversed multiple sclerosis in 17 of 21 patients; with none experiencing any deterioration in their conditions.

Q: But what about the FDA’s recent decision to allow a clinical trial using embryonic stem cells to treat patients with spinal cord injuries?

A: The FDA on 1/23/09 did give approval for the Geron Corporation to conduct a clinical trial using human embryonic stem cells to treat spinal cord injured patients. HOWEVER:

- ***This is not a trial to test the therapeutic efficacy of embryonic stem cell to treat spinal cord injury.*** It is more of an experiment to test the safety of injecting embryonic stem cells into human patients.

Q: Why is this necessary?

A: Because embryonic stem cells are know to form – often lethal – tumors in animal models.

- For this reason, even if the patients show no improvement from the trial, they will nonetheless have to be monitored for tumor formation for the rest of their lives.
- In addition, the patients will have to take immunosuppressant drugs to guard against rejection of the foreign embryonic stem cells.

Q: Given these risks, are there any alternative stem cell treatments that might be used to help spinal cord injured patients?

A: YES. ***Adult Stems have already been used to treat patients with spinal cord injuries*** and have been proven not only to be safe but also effective in providing some therapeutic benefit to the patients.

- A study from September 2008 by Australian researchers documented modest improvement in a spinal cord injured patient using the patient’s own adult nasal cells (“Autologous olfactory ensheathing cell transplantation in human paraplegia: a 3-year clinical trial,” Mackay-Sim A et al. *Brain* 131: 2376 - 2386, Sept 2008).

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- A June 2006 clinical trial in Portugal involved 7 patients, all of whom enjoyed improvements to their condition after being treated with their own adult stem cells, including increased movement and sensation, with some patients regaining some voluntary muscle movement and some bladder function. (“Olfactory mucosa autografts in human spinal cord injury: a pilot clinical study,” Lima C et al. *Journal of Spinal Cord Medicine* 29, 191-203, 2006). (Another patient who was treated by Dr. Carlos Lima, the lead researcher in this study, describes the benefits of her treatment at: <http://www.stemcellresearch.org/testimony/video/rabon.wmv>).

Q: With the FDA’s approval of the Geron trial, is there now a consensus that embryonic stem cells will prove the best way to treat spinal cord injured patients?

A: NO. Prior to receiving it, Geron had for many years been trying to get FDA approval for a clinical trial. When the approval was finally announced in January, skeptical voices and several concerns were raised:

- "There's a lot of debate among spinal cord researchers that the preclinical data itself doesn't justify the clinical trial," Neuroscientist Evan Snyder, director of the stem cell research center at the Burnham Institute for Medical Research, San Diego, CA. (“Celebration and Concern Over U.S. Trial of Embryonic Stem Cells,” Jennifer Couzin *Science* 30 January 2009: Vol. 323. no. 5914, p. 568).
- [W]e're still ... a long way from really understanding a good deal about these cells and how to use them safely," University of Pennsylvania’s John Gearhart, who led one of the first two teams that first isolated embryonic stem cells in 1998 (“Celebration and Concern Over U.S. Trial of Embryonic Stem Cells,” Jennifer Couzin *Science* 30 January 2009: Vol. 323. no. 5914, p. 568).

Q: If adult stem cells are already providing benefits to spinal cord injured patients, without the risks presented by embryonic stem cells, why use embryonic stem cells at all?

A: The first presidential bioethics commission to endorse federal funding for human embryonic stem cell research (in 1999 under then-president Clinton) declared such research “is justifiable only if no less morally problematic alternatives are available for advancing the research.”

- Adult stem cells are a real alternative to embryonic stem cells in helping patients. They have provided therapeutic benefits to patients with spinal cord injuries and for 72 other disease and conditions, without the risks posed by the use of embryonic stem cells, and without the moral baggage associated with the destruction of living human embryos to obtain stem cells.
HOWEVER:

Embryonic stem cells can be patented while a patient’s own adult stem cells cannot.

- "[T]his is not the most popular way of attempting to heal spinal injuries. That would be to produce patented chemicals, which drug companies can make and sell. What we're proposing could

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be carried out by any very modestly equipped hospital with neurosurgery. ***There are no patents. It makes it a very unpopular form of research... We're producing a procedure where the patient is their own cure. You can't patent a patient's own cells, thank God.***" London's National Institute for Medicine's Dr. Geoffrey Raisman, one of the world's leading researchers on the use of adult stem cells to treat spinal cord injured patients (*The Guardian* (London) 11/30/05).

Just as drugs are patented, so can embryonic stem cells – at potentially great profit for the biotech industry:

- “Geron Corp. (GERN) shares are soaring after the biotechnology company said it received FDA approval to begin trials of the world's first study of a treatment based on human embryonic stem cells aimed at spinal cord injury...Geron shares are up 35.5% to \$7.08 and are trading in a range of \$6.19 - \$7.18 in today's regular session” (“Geron Soars 35%, *Midnight Trader*, Comtex News Trader 1/23/09).
- They've [Geron] invested a ton of money and they never would have done that unless they had certain exclusive rights that have given comfort to investors putting money in the company" Carl Gulbrandsen, Managing director, Wisconsin Alumni Research Foundation (WARF) (“Stem Cell Therapy Cleared for Trials;” *Milwaukee Journal Herald*, 1/24/09).

THEREFORE:

- If the long term goal is to increase profitability by obtaining patents for the complex procedures that reduce the safety risks and produce usable embryonic stem cell-derived treatments that are also patentable, then the use of embryonic stem cells rather than adult stem cells becomes more preferable for the biotech industry.
- ***But if the goal is to put patients, not patents, first,*** and provide near term benefits but at lesser long term profits, then adult stem cells are, from a therapeutic perspective, clearly preferable.

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2/10/09