Where Research Ethics Meet Profit Margins

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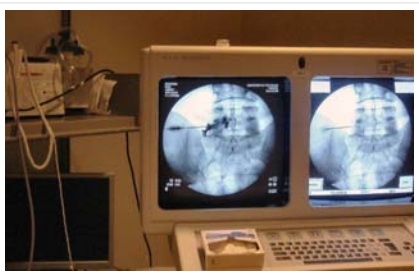
A biotechnology company's decision to end an experimental stem-cell study for business considerations raises important ethical questions.



Geron is a pioneering biotechnology company in the field of human embryonic stem-cell (hESC) research. It funded the first successful derivation of hESCs, announced in 1998. It also funded the first human safety study of hES-derived cells, which has been underway since 2010. A few days ago, to the surprise and consternation of many, Geron announced that it was quitting the business of stem-cell research and would not be enrolling any more spinal-cord-injury patients in its first-in-the world study of oligodendrocyte progenitor cells (GRNOPC1).

In July 2010, the U.S. Food and Drug Administration (FDA) approved safety testing of GRNOPC1 in 10 patients with subacute thoracic spinal-cord injury. These are patients who, as a result of traumatic injury, are paralyzed from the waist down, and agree to participate in research within two weeks of injury.

Geron enrolled its first patient in October 2010. T. J. Atkinson, a 21-year-old University of South Alabama Nursing student, was partially paralyzed as a result of a car accident that occurred on Sept. 25, 2010. The stem-cell transplant occurred on Oct. 8, 2010, at which time two million oligodendrocyte progenitor cells were injected into his spinal cord. The second spinal-cord-injury patient was enrolled in May 2011, and, since then, two other patients have been enrolled (the most recent enrolment taking place in September 2011). Geron's abrupt corporate decision to exit the field of stem-cell research and halt the GRNOPC1 trial leaves these four research participants in limbo. The ethical implications of this turnabout are cause for concern.

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The world is rife with speculation as to the reason(s) for Geron's dramatic decision to discontinue its stem-cell programs. It has been suggested that the oligodendrocyte progenitor cells being tested in patients may not be showing results as promising as originally hoped for, from either a biological or a business perspective. Some point to problems with trial design for the GRNOPC1 study, and with the choice of target population. Others point to recent developments with induced pluripotent stem (iPS) cells, and how these developments may have diminished the potential value of hES-derived therapeutics. (iPS cells are adult (somatic) cells that have been reprogrammed to function like embryonic cells, and, for many, these cells appear to be less ethically controversial.)

For its part, Geron insists that the decision to direct its human and financial resources to the development of its oncology drugs instead of its stem-cell products was made for purely business reasons, and that it does not reflect on

the safety or efficacy of the GRNOPC1 cells under study, or on the quality of other cell lines in its product portfolio. The company's news release states: "The decision to narrow Geron's technology and therapeutic focus was made after a strategic review of the costs, value inflection timelines and clinical, manufacturing and regulatory complexities associated with the Company's research and clinical-stage assets."

Geron says it will continue to follow the four patients currently enrolled in the trial and update the FDA on its research findings. Stephen Kelsey, Geron's chief medical officer, is reported to have said, "We will be reporting the results, and it will be a fair reflection of what would have happened if we had completed the study."

To some, this will seem an appropriate response to an unfortunate situation. I want to suggest, however, that this is an ethically problematic response to a situation that could have been anticipated, and thus avoided. Failure to name the problem in this way leaves future participants in safety studies of novel hESC interventions at risk of abandonment whenever a private biotech company decides to end a trial mid-stream for business reasons.

The original estimated enrolment for the FDA-approved trial was 10 participants. To date, only four participants have been enrolled. Geron now seems to be suggesting that the data from these four individuals will be equivalent to the data that would have been available had it completed the trial and enrolled all 10 patients.

If robust conclusions can be drawn from as little as four patients, then why would Geron and the FDA have estimated the original enrolment at 10? It is widely accepted that clinical trials should enrol the minimum number of patients required to generate new knowledge, so as to minimize the number of persons potentially exposed to research harms. Does it follow that Geron and the FDA were willing to expose more research participants than necessary to the risks of a first-in-human trial of an untested hES-derived cell product?

On the other hand, if robust data actually requires the enrolment of 10 participants, then closing the trial early – and only having data from four patients – means that those four patients will have been exposed to the potential harms of research participation without the countervailing potential benefit of new knowledge for the future treatment of spinal-cord-injury patients.

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In response to this critique, Geron might argue that its business decision to close the GRNOPC1 to further enrolment was not about increasing profits, but about avoiding bankruptcy. Were the latter to occur, the clinical trial would be halted and the participants would be no better off (and, arguably, they might be worse off). From another perspective, Geron might argue that business is business and, in a capitalist world, companies can decide how best to manage their financial and other resources to meet their obligations to shareholders.

This episode in the history of research involving humans suggests that, as more and more research is planned and executed in the private sector, it may be important for both regulators and research ethics committees, at the time of peer review, to ensure that companies funding safety trials have both the resources and the will to complete the trials they initiate. If there is any doubt about this, either approval should be denied or appropriate notice should be given to prospective research participants.

It is one thing for a company to cancel a clinical trial and halt enrolment because of problems with trial design, unexpected evidence of serious risk of harm to research participants, or bankruptcy. It is another thing altogether for a company to cancel a trial because there are other research avenues that might improve profit margins. Geron's news release, detailing the shift in focus away from its stem-cell programs in favour of its oncology programs, suggests that the decision to halt the GRNOPC1 trial falls into this last category.

**Update: A news report from Stanford University confirms that a fifth patient was enrolled in the Geron study just prior to Geron's abrupt announcement on Nov. 14 that it was discontinuing the trial. The fifth patient chose to undergo the procedure on Nov. 16 even after learning that the trial would be cancelled.*

Tags: *Business, Ethics, Experiment, Food and Drug Administration, Geron Corp, human embryonic stem cells, injury, Medical Research, Patients, Science, spinal-cord injury, Stem Cells, study, Technology*

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