**The Ethical Dilemma Of Patient-Funded Trials**

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A recent [article](http://news.nationalpost.com/health/trend-sees-patients-paying-for-their-own-clinical-trials-but-critics-raise-ethical-concerns) by Tom Blackwell, which appeared on the National Post website, caught my attention. In the piece, Blackwell notes there is a growing trend which has some critics alarmed: having patients pay for their participation in clinical trials.

In the article, Blackwell mentions [Regenastem Inc.](http://www.regenestem.com/), an international medical practice company. Regenastem’s focus is on using a patient’s own adult stem-cells to treat severe heart, lung, and circulatory problems. The therapies, billed as safe, simple, and non-invasive, are geared towards patients who have exhausted the possibilities of other treatments.



Many readers will remember Detroit Red Wings hockey legend Gordie Howe generating a lot of media attention in late 2014 when he went to Mexico to undergo stem-cell therapy. Howe suffered a series of mini-strokes along with a serious one in October 2014 that left him bedridden. In a statement, Howe’s family noted they were contacted by Stemedica Cell Technologies, a biotech firm manufacturing stem cells derived from fetal brain tissue. (This aspect of the treatment apparently did not receive a lot of attention because the manufacturer refers to the cells as “adult” stem cells, due to the fact that they are considered more mature than embryonic stem cells).

The company offered to facilitate Howe’s participation in a stem cell trial using the experimental treatment at Novastem, a product distributor in Mexico. At the time Howe was bedridden with little ability to eat or communicate.

The two-day treatment was nothing short of miraculous. After day 1 Howe was walking with minimal effort for the first time since his stroke. By the end of day 2 he was conversing with family and staff. On the third day he boarded a plane under his own power. After a second round of treatments in June, Howe was enjoying the sunshine on a beach in San Diego.

What many did not know about the trial at the time is that other stroke patients participating in the study paid $32,000 to be part of it.

**Should Patients Pay For Trials?**

Regenastem has now secured lab space in an incubator in Ontario. For two upcoming trials conducted with a research company in Buffalo, NY, the company will pay for the trials using a patient-funded model. Blackwell notes many developers of new treatments, unable to access research dollars from governments, health charities, or pharmaceutical companies, are looking for financial support from the people who want to participate in the studies.

Obviously, the idea is generating some controversy. Critics warn these studies present ethical issues and may produce or be based on science that some might consider to be questionable and that could harm patients. Jonathan Kimmelman, a McGill University bio-ethicist, thinks we will see more of this in coming years for non-placebo-based studies. Although most trials are conducted by testing a new treatment against a blinded control group receiving a placebo, Kimmelman doubts patients will be willing to pay to be part of a study where they might receive a placebo.

One solution might be to charge patients upfront, and then later reimburse those receiving the placebo. That might work for treatment options that address some non-life-threatening condition. Unfortunately, for patients like Howe who have exhausted other options and now have little hope or time, a placebo study may not be a viable alternative. Kimmelman notes patient-funded studies may also be nothing more than a way for companies to test and market unproven treatments.

It’s no secret to anyone reading this article that clinical trials are the costliest aspect of drug development. It can also be the most time consuming part of the discovery process. In those instances where the treatment is for a rare disease, patient funding could pick up the tab for research that pharma companies and governments are not willing to support.

However, many would argue that pharma companies perform a lot of research and scientific rigor when determining which molecules are worthy of entering the clinical trial process. In a patient-funded model, desperate patients, and the size of their bank account, could be making those decisions.

If you’re wondering about the regulatory implications of patient-funded trials, Blackwell states U.S. law specifically allows for them in certain circumstances. For example, a company in Florida has received FDA approval to run a patient-funded erectile dysfunction study using stem cells.

**Ethical Dilemmas Will Arise**

As these studies continue to take place worldwide, one thing is certain: the controversy will likely not go away. Pharma and governments may not be willing to pay for these studies. And payers are unlikely to support experimental treatments that have not been tested in clinics or approved by the FDA. If that leaves payment in the hands of patients, it has the potential to bring up other ethical dilemmas.

I have had numerous discussions with friends in the past over the sale of organs. If a person has two healthy kidneys and is willing to sell one to someone who needs it, should the government allow the transaction? On the one hand, these types of transactions could go a long way towards solving the perpetual organ shortage problem. At the same time, the ethical dilemma it would present is the rich being made healthier off the backs (and organs) of the poor.

If the cost of a potentially life-saving treatment is $32,000, who would have that much money sitting idly in a savings account? Does that mean only the rich be able to afford such treatments? And what will these companies do the first time a child is in need of the treatment, but his/her parents are living below the poverty line?

It may be time for pharma to start considering the ramifications of such treatment options. Kimmelman would argue that such studies are currently more of a liability than an opportunity. An independent vetting of these trials might be the first step towards ensuring they will contribute to our library of knowledge, and not simply line the pockets of clinics performing these studies.