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The darker side of stem cells

An investigation by Nature *has found that patients in Texas are receiving unproven stem-cell treatments. The state and the company involved need to ensure that they follow FDA guidelines.*

Stem cells offer the hope that one day they will be able to cure a huge range of disorders. But too many people are promising those cures to patients now, long before there is any evidence that they work. These claims are potentially misleading at best, and at worst could be downright harmful.

This week, *Nature* raises important questions about one company that works with adult stem cells: Celltex Therapeutics in Houston, Texas. *Nature*'s investigation, reported on page 13, suggests that the company has supplied adult stem cells to Texas doctors who offer unproven treatments to patients, and that the company is involved in these treatments. One doctor claims that the treatments are part of a clinical study run by Celltex and that the company pays him US$500 a time to inject the cells into patients, who are charged up to $25,000 for a course. The US Food and Drug Administration (FDA) considers it to be a crime to inject unapproved adult stem cells into patients. David Eller, chief executive of Celltex, denies that the company is involved in treatment procedures, but would not comment on *Nature*'s findings about how its cells are used or answer questions about them.

Celltex has the backing of state governor Rick Perry, who has tried adult-stem-cell treatments himself. And the company recently recruited Glenn McGee, editor-in-chief of the *American Journal of Bioethics*, to be its president of ethics and strategic initiatives. McGee, whose move from the academic world to industry sparked a controversy over a perceived conflict of interest (see *Nature* **482,** 449–450; 2012), promises that Celltex will set up and run clinical trials, and will do so according to strict ethical standards. But he too would not answer *Nature*'s questions on whether the company knew about the unapproved clinical use of its stem cells or whether he considered such activity to be ethical and legal.

There is an ethical paradox here. How can Celltex propose clinical trials for stem-cell treatments while at the same time it is, according to a doctor involved, paying physicians to use those treatments — or supplying cells to doctors who would no doubt use them — in the clinic? Shouldn't clinical trials be done before a treatment is given to paying patients? Conversely, if a treatment is known to be safe and effective enough to be prescribed routinely, and for a sizeable fee, what is the point of doing a costly clinical trial?

The questions do not stop at Celltex. The governor-appointed Texas Medical Board is set to launch regulations in April to tighten controls on the use of 'investigational agents' — such as stem cells that do not have FDA approval. But the board is simultaneously offering an alternative route by proposing that those who want to use adult-stem-cell treatments need either FDA approval or simply the approval of a local institutional review board.

Texas officials should take the FDA's regulatory power over stem cells more seriously. In its discussions, the state's medical board revisited the well-trodden ground of whether adult-stem-cell procedures — in which cells are taken from a patient, processed and cultured, before being reintroduced — should be under FDA jurisdiction, or whether they are akin to simple skin grafts from one part of the body to another, which do not require validation in an FDA-approved clinical trial.

The FDA can help to clarify these matters. A sensible first step in the new state regulations would be a requirement for any firm that plans to inject processed stem cells into patients to contact the FDA, which can advise on whether federal rules — the same federal rules that have already been used to arrest stem-cell practitioners and to stop a company pushing unapproved treatments elsewhere — apply to what they are doing. Once past that step, Texas could move on to develop its own safety regulations.

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If the Texas Medical Board were to act according to its stated pledge to protect patients, then it would make clear the need for clinical validation of adult stem cells before use and would rescind the medical licences of any doctors in breach of rules on using unapproved treatments. If Celltex truly wants to help patients, then it should refuse to supply stem cells for medical procedures until those procedures are properly proven to be effective. And if the company is serious about demonstrating clinical effectiveness itself, then it should start by contacting the FDA about what needs to be done. ■

The great beyond

Progress on rare genetic diseases shows the medical value of outliers.

"Treasure your exceptions! When there are none, the work gets so dull that no one cares to carry it further. Keep them always uncovered and in sight. Exceptions are like the rough brickwork of a growing building which tells that there is more to come and shows where the next construction is to be." Geneticist William Bateson offered this advice in 1908, around the dawn of modern genetics following the rediscovery of Gregor Mendel's pea plant experiments, and it remains sound today.

Every empiricist must contend with exceptions to the rule, which can illuminate research in unpredictable ways. Bateson was urging vigilance in observing rare offspring of plants and animals, which may point to new phenomena that can inform us about the broader biological context. The same is true for rare diseases, which often have a genetic basis.

This being a leap year, the extra day, 29 February, was designated Rare Disease Day. It aims to give a voice to the families of millions of exceptional children who are born each year with a rare or undiagnosed disease. Liddle syndrome and Tangier disease, for example,