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News

FDA to vet embryonic stem cells' safety

US agency holds first public hearings to assess therapies.

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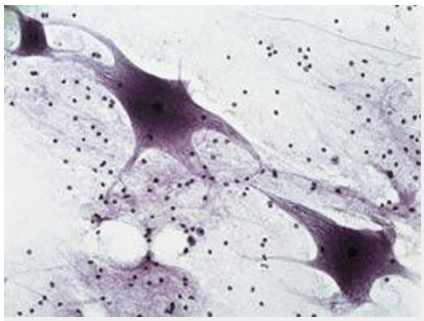

Geron plans to use human embryonic stem cells to treat patients with acute spinal-cord injury.

M. KAGE/SPL

Investors, biotech companies and other stem-cell stakeholders are meeting in Gaithersburg, Maryland, this week for the US Food and Drug Administration's (FDA's) first public hearing on the safety of therapies that use human embryonic stem cells.

The meeting is "a big deal", says Michael Werner, former chief of policy at the Biotechnology Industry Organization in Washington DC. "It could provide clues about what the FDA is thinking in terms of product approvals."

Stem cells derived from human fetal and adult tissues are already being used in clinical trials. But researchers want to use embryonic stem (ES) cells because they show greater capacity to proliferate and differentiate into other cell types. Cells derived from human ES cells have shown dramatic results in treating animal models of disease, but they have not yet been tested in patients, and there are fears that they may carry health risks.

Geron, a biotech company based in Menlo Park, California, plans to start a trial in patients with acute spinal-cord injury this summer using oligodendroglial progenitor cells derived from ES cells. Two other California-based biotech companies, Novocell in San Diego and Advanced Cell Technology in Los Angeles, are also preparing to start human trials using ES-cell-derived products to treat (respectively) diabetes and visual impairment caused by macular degeneration.

The FDA seems to be most concerned about the cells' potential to cause tumours or to differentiate in dangerous ways, and whether the animal safety tests that have been carried out so far provide enough evidence to justify testing ES cells in people, according to FDA briefing documents seen by Nature. Another issue concerns how patients should be monitored for signs of problems. Members of the advisory committee have been asked not to speak to the press before the meeting.

The FDA has been looking at these issues for a number of years, according to Michael West, head of BioTime, a biotech in Emeryville, California, and a former executive of Geron and Advanced Cell Technology. "The first time I met with them was in 2001 and they had given it a lot of thought back then," he says.

Human trials will not administer actual ES cells, but cells derived from them, and one of the biggest issues is how to assess the final cell product. One question is how many partially differentiated or undifferentiated cells, if any, are acceptable, and whether undesirable cells can be reliably detected. "You might have cells destined for the spinal cord mixed in with precursor cells destined to make a wisdom tooth," West says. "What happens when you put those cells into the spinal cord?" West says he's not against clinical trials, but he points out that the FDA has a very difficult balance to strike.

No decisions will be made at the meeting, but the process should eventually lead to guidelines from the FDA about required preclinical studies, trial design and patient follow-up, says Marie Csete, chief scientific officer at the California Institute for Regenerative Medicine (CIRM). This will be of great interest to investors, who hope to gain clues about the FDA's expectations. "The greater clarity the FDA can provide around those kinds of rules the easier it is for me to evaluate [ES cells] as an investment opportunity. Right now it isn't clear," says Gregory Bonfiglio of Proteus Ventures, which invests in regenerative medicine companies.

Csete describes the meeting as "a necessary first step" in bringing stem cells to the clinic within a well-regulated environment. CIRM, she says, will expect investigators it funds to implement scientific plans that lead to clinical trials, and she hopes this meeting will indicate what in vitro and animal work should be done. "We want to make sure that all the appropriate studies are considered that assure that human ES-cell-derived cell products are optimized for function and safety."

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