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Stem Cells: Blindness, Yes; Parkinson's, Probably Not

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I spent yesterday afternoon and evening at the annual meeting of the New York Stem Cell Foundation, at Rockefeller University, where the organizers were firm taskmasters: the scientists presenting studies had to focus not on esoterica but on translational research—that is, the kind that promises to help patients.

Two presentations stood out. Pete Coffey of University College London described the work he is doing as part of the London Project to Cure Blindness, which has made remarkable progress using human embryonic stem cells (donated by couples using IVF who had "extra" embryos they did not wish to implant) to treat the blindness caused by macular degeneration). You read it here first: in the magazine's "global literacy" issue in July I argued that the first clinical use of stem cells would be to treat blindness. The work Coffey described makes that seem more and more likely: he and his team have improved the vision of several patients who had gone blind. One of them was even able to drive again.

But Coffey was quite circumspect: the surgery so far works on only 25 percent of patients. He and his team continue to refine the technique and define the patient populations that would most benefit from the surgery.

That good news stood in stark contrast to the talk by Jeffrey Kordower of Chicago's Rush Presbyterian Medical Center, where he is a leading neurologist. His task was to describe progress on using stem cells to treat Parkinson's disease, which on paper looks like an excellent candidate for the stem-cell approach. Scientists know what has gone wrong in PD. Neurons in the brain's substantia nigra die or do not work; either way, they fail to produce dopamine, leading to the tremors and other symptoms of PD.

Solution: replace them with neurons derived form stem cells, or with stem cells themselves that you coax to differentiate into the lost/damaged neurons. I've blogged before on the growing realization that using stem cells, or cells derived from stem cells, to repair neurological diseases is going to be really, really hard. But I'd never heard someone of Kordower's stature put it quite so starkly: "In my opinion it will take a major miracle for stem cells to make a difference in Parkinson's disease," he said.

In fairness, as he went on to say, the field of PD has already had two miracles: the discovery that the drug levo-dopa and the technique of deep-brain stimulation both dramatically reduce the movement symptoms of PD. But attempts in the 1990s to transplant fetal brain cells into PD patients produced awful side effects (uncontrolled movement, called dyskinesia), for no-one-knows-what reason. "No one should do clinical trials with stem cells [for PD] until we understand the cause of the dyskinesia," Kordower warned. Another cautionary note: in earlier studies, the transplanted cells eventually show the same kind of awful changes that the patient's own brain cells did, such as loss of dopamine transporter and development of Lewy bodies, a cause of dementia. In other words, whatever went wrong in the brain originally to produce Parkinson's was still going wrong, ravaging the transplanted cells. Very bad news for Parkinson's patients who have pinned their hopes on stem cells—and ironic given the prominent, courageous role Michael J. Fox and his foundation have played in drumming up public support for stem cell research.