

Where Are the Cures?

IT HAS BEEN YEARS SINCE HANS KEIRSTEAD WORKED HIS BIOLOGICAL MAGIC, injecting stem cells into rats with severed spinal cords and thus making them walk almost normally. But the real miracle—since other experiments, too, have cured paralysis in lab animals—is that Geron Corp. plans to test the technique in people next year. Between Keirstead's experiment and Geron's trial lie these obstacles: Keirstead, a professor at the University of California, Irvine, had to invent

instruments to squirt the stem cells into spinal cords ("what do we academics know about developing medical devices?" he asked me), find someone to try the technique in monkeys ("I know two researchers who handle monkeys; you have to get in line"), ramp up production of the stem cells ("it meant going from pipettes to this massive hydraulic setup") and ... well, more industrial-strength biology that he wasn't trained in, that the government rarely funds and that brings exactly zero glory to a university scientist. "We hacked through the jungle and paved the road," Keirstead said. "But how many others are willing to do that?"

Going by how few discoveries in basic biomedical research get turned into treatments and cures, the answer is very, very few. The nation's biomedical funding and training system are set up to do one thing, and they do it superlatively: make discoveries. That is what scientists dream of, that is what gets them published in leading journals (the coin of the realm in academia) and that is what gets them grants from the National Institutes of Health. Here's what doesn't get them any of those: the grunt work that Keirstead did to turn his spinal-cord breakthrough into something that can be tried in patients.

These barriers to "translational" research (studies that move basic discoveries from bench to bedside) have become so daunting that scientists have a phrase for the chasm between a basic scientific discovery and a new treatment. "It's called the valley of death," says Greg Simon, president of FasterCures, a center set up by the (Michael) Milken Institute in 2003 to achieve what its name says. The valley of death is why many promising discoveries—genes linked to cancer and Parkinson's disease; biochemical pathways that ravage neurons in Lou Gehrig's disease—never move forward.

The next administration and Congress have a chance to change that, radically revamping the nation's biomedical research system by creating what proponents Richard Boxer, a urologist at the University of Miami, and Lou Weisbach, a Chicago entrepreneur, call a "center for cures" at NIH. The center would house multidisciplinary teams of biologists, chemists, technicians and others who would take a discovery such as Keirstead's and nurture it along to the point where a company is willing to put up the hundreds of millions of dollars to test it in patients. The existence of such a center would free scientists to go back to making important discoveries, not figuring out large-scale pipetting, for goodness' sake. "There is a sense among dis-

ease philanthropies that something like this is what needs to happen," says Katie Hood, CEO of the Michael J. Fox Foundation for Parkinson's Research. "It's a huge opportunity for a new administration."

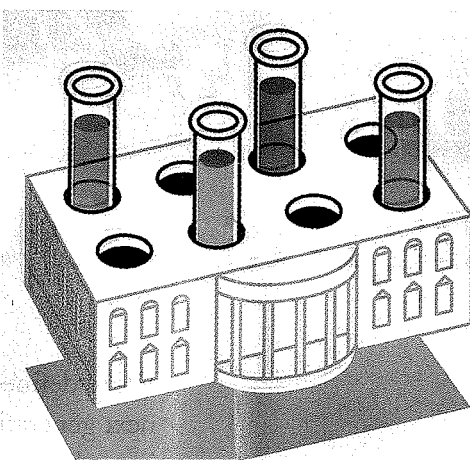
Biomedical scientists I spoke to are wary of using NIH money for a new center for cures. They worry that it would divert dwindling funds from the basic research that is their pride and joy and, indeed, the basis for those hoped-for cures. Given current fiscal realities, scientists are right to be worried. But while basic research is necessary for finding new treatments, it is

not sufficient. (While the NIH budget was doubling, the number of new-drug approvals fell from 53 in 1996 to 18 in 2006.) When I asked Kierstead if he ever wondered how many promising leads are gathering dust between the covers of research journals because no one is willing or able to push them forward, he said, "I don't wonder. I know it's the case." Why? Because "curing disease is a byproduct of the [NIH] system and not a goal," says FasterCures' Simon. Most scientists don't want to and don't have the skills to translate a discovery into a treatment; researchers at a dedicated center would try to do that full-time.

Some disease foundations have paved the way, turning themselves into mini-centers for cures. The pioneering Myelin Repair Foundation, which funds research on treatments for multiple sclerosis, actively manages the five scientists at five universities whom founder Scott Johnson hand-picked, requiring them to share data almost as fast as they collect it, mandating collaboration and pushing discoveries through the valley of death. For instance, a test-tube finding is quickly

tested in a mouse model; contractors are hired to develop ways to scale up a discovery of how to turn stem cells into myelin-making cells that could help MS patients.

There is lots of talk these days about increasing the nation's spending on infrastructure, such as roads and bridges, to lift the economy out of its doldrums. Me, I'd be willing to put up with potholes in exchange for a new administration spending serious money to take the discoveries taxpayers *have paid for* and turn them into cures.



Scientists call the gulf between a biomedical discovery and new treatment 'the valley of death.'