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HEALTH CARE

REPORT

Field of vision

Private NY lab becomes major player in stem-cell research

BY GALE SCOTT

Stem-cell research is a sizzling-hot field—the box-office blockbuster of biomedical research. Global competition for funding and scientists is fierce, and the stakes—the potential to use these human cells to cure deadly diseases, restore sight, mend broken bones, even reverse mental decline—are enormous.

Those prospects are so exciting that Californians voted in 2004 to spend \$3 billion over 10 years to set up a stem-cell research institute. The global research tab is in the hundreds of billions of dollars, while the National Institutes of Health spent nearly \$3.5 billion from 2008 to 2011. The NIH expects to spend \$1.2 billion this fiscal year, about what it has spent in each of the past two years.

But in a small laboratory in Manhattan, using mostly private donations, the upstart New York Stem Cell Foundation has become a major player in just six years. Starting with three employees and no grant money, it has grown to a staff of 51, boasting affiliations with a dozen academic centers and an annual budget last year of \$18.3 million. Its scientists' success in introducing DNA from a human skin cell into an unfertilized egg from a separate donor was named the "No. 1 medical breakthrough of the year" in 2011 by *Time* magazine.

And in a second remarkable achievement, foundation researchers have devised a method for culturing hundreds of living human cells in less time than it takes to grow them one at a time—a milestone in the quest to find a way to screen new drugs quickly and safely.

"NYSCF has been astonishingly successful," said Robin Elliott, executive director of the Parkinson's Disease Foundation. "They've done an amazing job in creating a major institution in New York at a time when biotech money has been very scarce."

Founder Susan Solomon, a successful Manhattan businesswoman

\$100M
AMOUNT
NYSCF has
raised since
its founding
in 2005



SUSAN SOLOMON
created the New
York Stem Cell
Foundation with
her own money.

BUCK ENNIS

with a law degree and an entrepreneurial bent, created it with her own money in 2005. The idea came to her after she had been through a string of unhappy family events, including her mother's death and a son being diagnosed with diabetes. "Everyone experiences their mortality differently," she said. "I started thinking about curing some of these terrible diseases, and I started this institution."

Ms. Solomon rented a 500-square-foot lab and did fundraising from her apartment. Already an experienced fundraiser for the Juvenile Diabetes Foundation, she talked with scientists who, she learned, were angry about federal restrictions on stem-cell research. The Bush administration had banned using federal funds to create new stem-cell lines from discarded human embryos. Scientists told her they feared that medicine had lost the work of a generation, as young scientists chose other fields or decamped to countries without such restrictions. Researchers were eager to see her succeed and loved the idea of a private lab free from government interference.

Since then, the foundation has weathered the controversy over the use of embryonic stem cells and has raised \$100 million, including more than \$90 million from private sources and \$8.4 million from New York state. It collaborates actively with other institutions, training and supporting researchers or working with clinicians at Rockefeller University, Memorial Sloan-Kettering Cancer Center, Mount Sinai Hospital, NYU Langone Medical Center, Albert Einstein College of Medicine and New York-Presbyterian Hospital and its affiliated medical schools.

Contributing to progress

"We are trying to be a catalyst," said Ms. Solomon. "Our whole focus is on accelerating the field, and we're agnostic as to where an advance comes from."

And though NYSCF has yet to find a cure for any of the targeted diseases, its discoveries are widely seen as contributing to significant progress. Ms. Solomon credits that achievement to her decision to give researchers, most of them newly minted physicians and Ph.D.s, free rein to explore their interests and hunches without the academic or governmental constraints other scientists face. "We're small and very entrepreneurial," she said. "If something makes sense, we can just do it."

That freedom to work on whatever disease or scientific puzzle interests them is a big draw for young scientists like Mike Nestor, 34, who is an expert in electrophysiology, the study of the electrical properties of cells and tissue. Mr. Nestor came to NYSCF after a three-year stint at the National Institutes of Health. Theorizing that the memories lost in Alzheimer's disease are not truly lost but are stored in the brain's hippocampus, he is using

his knowledge of electricity to find a way to relight the brain circuitry shut down by the disease.

"I'm here so I can do my experiments," he said. "At the NIH, my hands were tied because your work has to follow the government's dollars," as well as the interests of one's superiors.

Though the foundation has a full-time staff, including Dieter Egli and Scott Noggle—the superstars credited with the discovery that made the cover of *Time*—most of those bent over microscopes or monitoring high-tech instruments have three-year fellowships and are in their early 30s, just starting their careers.

Postdoctoral fellow Giuseppe Maria de Peppo, 30, is using stem cells to try to grow new bone tissue on a matrix of cells from cadaver donors. Others are working on replicating cells damaged by multiple sclerosis, Parkinson's disease, leukemia, ALS and other debilitating, often-fatal maladies. Still others are focused on the delicate and critical mechanics of manipulating stem cells.

Young staff

Though many institutions are engaged in medical research, the New York foundation is unique. "They may well be the single biggest [institution] to focus 100% on stem cells," said Brock Reeve, executive director of Harvard Stem Cell Institute, which has collaborated with NYSCF.

Not that there aren't challenges ahead. Cures for the myriad ailments in which scientists believe stem cells may play a role are likely years, even decades, away. In the meantime, the foundation has to find new funding every year. Recent donors include the Charles Evans Foundation, which gave \$1 million for work on Alzheimer's disease. But the facility has no income-generating endowment or federal funding.

Although the 2001 ban did not completely eliminate federal funding for stem-cell work, it limited researchers to cell lines created before that year. Since then, guidelines issued in 2009 allow researchers to work on new lines of embryonic stem cells developed with private funds. That vastly expanded the number of cell lines available. But the ban on using federal funds to create these lines remains in effect.

The result is that much of the work done by NYSCF is still not eligible for federal money, and its researchers must rely almost entirely on private donors. Unlike hospitals and disease-centric foundations, NYSCF can't appeal to appreciative patients and families—at least not yet.

"We don't have grateful patients; we have hopeful patients," said Ms. Solomon. "It's harder, and it takes someone who is a little more sophisticated and who understands how medical research works." ■

