



## **Stem Cell Research in New York State Fosters Job Creation and Medical Innovation**

### **Background**

In 2002, the federal government severely limited federal financing for embryonic stem cell research, a move that set off intense competition among states to create publicly funded stem cell programs. Although the Obama Administration has expanded the number of stem cell lines available for use in NIH funded research from 21 to 86 lines, limitations still exist. As a result, federal funding restrictions, like the derivation of stem cell lines, have prompted states to establish state-based research funds aimed at capturing the scientific and commercial potential of this new field of medicine.

In 2004, New Jersey and California were the first states to establish state funded stem cell programs. Subsequently, to remain competitive, New York created its own publicly financed stem cell program.

In the spring of 2007, New York dedicated \$600 million over 11 years for stem cell research, making it one of the largest government-financed stem cell programs in the country. The State's objective was to sponsor a strong research community in New York State that could investigate the potential of stem cell technology to alleviate disease and improve human health. Of near equal importance was the goal of economic development – the investment in research and facilities that would create jobs, both directly and indirectly, and to fuel the local economy. The Empire State Stem Cell Board (“Board”)

was also created and charged with making grants for basic, applied, translational, and other research and development activities that will advance stem cell research throughout the State.

New York's funding commitment is critical to the State's stem cell research community given its unique nature. In addition to the derivation of stem cell lines, New York's program is also unique given that it funds early stage research projects that have had difficulty accessing other funding sources, like the National Institutes of Health ("NIH"). New York's program also provides funding for capital and equipment to create a stem cell infrastructure.

Because of New York's funding commitment, funding has allowed the state's biomedical research and commercial sectors to grow, and has placed New York at a scientific and economic advantage over state's that do not have dedicated stem cell programs.

Since the beginning of 2008, the Board has allocated nearly \$223 million to support stem cell scientists in the development of new research, training, collaboration and research infrastructure. New York's medical schools, the New York Stem Cell Foundation (NYSCF), and Memorial Sloan-Kettering Cancer Center have been awarded over 55 percent, or \$158 million, in total funding to date. The remainder has been awarded to other health care and research institutions, including the Regenerative Research Foundation and Cold Spring Harbor Laboratory. This funding commitment positions New York as a leader in stem cell research, and brings hope to millions of people suffering from a range of debilitating diseases. Scientists say that in the future, stem cells may be used to replace or repair damaged cells and have the potential to drastically change the treatment of conditions like Alzheimer's disease, amyotrophic lateral sclerosis (i.e. ALS or Lou Gehrig's disease), burns, cancers, spinal cord injury, Parkinson's disease, juvenile diabetes and other conditions.

New York's stem cell program has been extremely successful since its inception and continues to foster medical innovation and job creation throughout the State. The infusion

of state funds for stem cell research has been “catalytic” for New York. The state investment made includes a big multiplier effect as setting up a center or consortium leads to a significant amount of external funding, which creates jobs and fuels the local economy. This has enabled New York to become more competitive for federal and philanthropic funding for research.

### **Medical Innovation**

Stem cells are self-renewing and generate other cells in tissues. Given their role in development and regeneration of tissue, stem cells are at the core of developmental biology and clinical applications.

Many medical schools and research institutions from across the State are conducting cutting-edge stem cell research funded partially by the New York Stem Cell Board.

Examples include:

*At the Albert Einstein College of Medicine at Yeshiva University, researchers are working to treat and cure diseases ranging from cancer and anemia to heart and liver diseases, obesity, and brain disorders. In addition, Einstein scientists are doing some highly advanced work on replicating liver cells that could reduce the need for liver transplants using live donors and cadavers, which are in short supply. This research could also lead to treatments for genetic diseases such as hemophilia.*

*Columbia University Medical Center researchers have laid the groundwork for possible future joint replacement for patients with diseased or damaged joints. This pioneering research, carried out at the Columbia University Medical Center in conjunction with colleagues from the University of Missouri and Clemson University, paves the way for future joints that would last longer than today’s artificial joints. To set about growing a new joint, researchers first laser-scanned the surface contours of a hip joint in an animal*

model and then used a special bio-printer to create a three-dimensional life-sized scaffold upon which cartilage and bone could be regenerated. Then, using a unique stem cell homing technique in the animal model, researchers orchestrated the body's own stem cells, without removing them from the body, to migrate to the scaffold. Growth factor infused in the scaffold helped the stem cells grow into a functioning, native "synovial" joint. An estimated 400,000 people each year receive an artificial joint to replace a knee or a hip, and could potentially benefit from this type of research.

At the *Mt. Sinai School of Medicine*, researchers have for the first time differentiated human stem cells to become heart cells with cardiomyopathy, a condition in which the heart muscle cells are abnormal. The discovery will allow scientists to learn how those heart cells become diseased and from there, they can begin developing drug therapies to stop the disease from occurring or progressing. The study is published in the June 9th issue of *Nature*.

The Mount Sinai team used skin cells from two patients with a genetic disorder known by the acronym LEOPARD syndrome. Hypertrophic cardiomyopathy, or thickening of the heart muscle, is experienced by 80 percent of patients with LEOPARD syndrome and is the most life-threatening aspect of the disorder. The Mount Sinai team took patient skin cells and reprogrammed them to become pluripotent stem cells. Such cells can then develop into almost any type of cell in the human body. The researchers then created heart cells that had characteristics of hypertrophic cardiomyopathy.

At the *New York University ("NYU") College of Dentistry*, a dental research team is studying the potential use of stem cells to regenerate facial muscles to enable people with traumatic injuries to speak, eat, and smile normally again. The team plans to extract a small number of stem cells from the facial muscle of a pig, grow the cells into facial muscle on a tissue scaffold to develop a prosthesis, and transplant the prosthesis onto the pig's face to repair damaged muscle. Pigs are being used for the new study because their extracellular matrix is similar to that of humans, and because successfully transplanting the stem cells in a large animal model would pave the way for a clinical trial on humans.

*SUNY Upstate Medical University* is unique among universities throughout the United States in having a center dedicated to "Humanized" SCID Mouse (SCID-HU mouse) research. SCID (severe combined immune deficient) mice are genetically engineered mice that lack immune systems. Without their own immune systems, the mice can accept transplants of human hematopoietic stem cells (HSCs) that then mature and develop into all components of the human immune system, generating "humanized" SCID mice. Thus, they are vital for research into human viral infections, cancer stem cell biology and in translational development of anti-viral drugs and therapies for treating hematological disorders, scientists say.

Stem cell research at SUNY Upstate is focused primarily on developing a "humanized" mouse model to study the initiation and progression of leukemia induced by human viruses, specifically Adult T cell leukemia, Burkitt's lymphoma and primary effusion lymphoma. Other models of infection and disease to be studied in humanized mice include the chicken pox virus, malaria, hemorrhagic and encephalitic viruses, and Kaposi's Sarcoma herpes virus.

At the *University of Buffalo State University of New York (SUNY) School of Medicine and Biomedical Sciences*, a biochemist in UB's New York State Center of Excellence in Bioinformatics and Life Sciences and his colleagues have received \$3.5 million from the Empire State Stem Cell Board to establish a Western New York Stem Cell Culture and Analysis Center. The funds will be used to promote and facilitate research in the use of mouse and human embryonic, adult, induced pluripotent and cancer stem cells. Pluripotent cells have the ability to become nearly any type of cell in the body.

At the *University of Rochester*, scientists have been awarded grants to further research in neurological disease, cancer, cardiovascular disease, and bone repair. Specific projects include research to treat Huntington's disease and other neurodegenerative disorders, research into treatments for brain cancer, leukemia, and colon cancer, treatments for pulmonary disease, and using state-of-the-art imaging technology to evaluate treatments for bone repair and regeneration.

At *Weill Cornell Medical College*, researchers received a state grant with the overall aim to establish new, as well as maintain and support existing multi-institutional core facilities at the Rockefeller University (RU) and Weill Cornell Medical College (WCMC). These two neighboring institutions are among the world leaders in stem cell biological research and enjoy a long lasting tradition of collaborative research. As such, the facilities will undertake a comparative approach between human and mouse stem cells (embryonic and adult) in their ability to differentiate into various specialized cell types. The derivation of novel human embryonic stem cell lines (hESC), reprogramming of somatic cells and differentiation into all three embryonic germ layers -- ectoderm, mesoderm and endoderm – will allow researchers to develop specific cell-based therapy platforms and target specific diseases affecting: 1) the nervous system; 2) the skin; 3) the vasculature and blood; and 4) the liver and pancreas. These strategies should enable researchers to develop novel cell-based therapies and technologies that open novel avenues in modern medicine.

Researchers at *Memorial Sloan Kettering Cancer Center (MSKCC)* are studying the mechanisms of stem cell self-renewal and pluripotency. The fundamental mechanisms that control the behavior of stem cells remain poorly understood. MSKCC investigators are engaged on multiple fronts in an effort to explore basic questions related to stem cell self-renewal and differentiation, the acquisition and maintenance of pluripotency, the development progression of stem cell fates, and the properties of the stem cell niche.

MSKCC investigators are also actively studying stem cells and cancer. Insights from stem cell biology can provide a novel understanding of the mechanisms that underlie tumor biology. In particular the study of stem-like cells within tumors may spur the development of new therapeutic approaches aimed at therapy-resistant populations of tumor cells. Access to human stem cells and precise tools for genetic manipulation enable a new generation of human stem-cell based models of cancer. Such human cancer models can complement animal studies and may result in novel strategies for drug discovery.

At the *New York Stem Cell Foundation* (“NYSCF”), researchers secured nearly \$1 million from state funds to establish an efficient nuclear transfer protocol that allows the routine derivation of stem cell lines using somatic cell nuclear transfer (“SCNT”), a research technique that is not allowed to be done with federal funds.

The NYSCF also received a NYSTEM grant to develop new human embryonic stem cell lines from embryos diagnosed genetically to have either Spinal Muscular Atrophy or Huntington’s disease and make models of these diseases in a dish. Currently, the NYSCF is obtaining embryos from In Vitro Fertilization (“IVF”) clinics that are pre-genetically diagnosed to contain the diseases and then deriving cell lines to study in a dish and test drugs on. The NYSCF also received a shared facility grant from state stem cell funds to expand its existing multi-institutional research center to provide researchers with equipment, technologies, and expertise for stem cell derivation and characterization that would otherwise not be available to scientists without this state funded grant. Research projects for this grant include Alzheimer’s disease, Parkinson’s disease, diabetes, cardiac repair, bone regeneration, spinal muscular atrophy, Huntington’s disease and schizophrenia.

### **Economic Contributions**

In addition to supporting ground-breaking stem cell research projects, the state’s investment in these projects has created new jobs and is attracting top researchers to New York. Leading scientists and medical professionals from across the country are coming to New York because they are able to conduct cutting-edge research in the State. In doing so, these scientists are often bringing with them their National Institutes of Health (“NIH”) grants and post doctoral students. Furthermore, medical schools and research laboratories are hiring new researchers to compliment the stem cell programs. The growing research infrastructure brings increased revenue for research facilities and staff and the ability to train new graduate students, develop new drug therapies, and spin off clinical businesses throughout the region.

In total, over 300 jobs have been identified as being created in just over two years specifically as a result of New York stem cell research funds. Moreover, these jobs have led to a multiplier effect creating hundreds of additional jobs as a result of initiated “seed” stem cell funding. Specific examples include:

*At the Albert Einstein College of Medicine, 12 new researchers have been hired.*

*At Columbia University Medical Center, for example, an estimated 100 positions have been hired and/or maintained with state funding.*

*At New York Medical College, three post-doctorate fellows and two new faculty members were hired.*

*At SUNY Upstate Medical University, an estimated six new researchers have been hired.*

*SUNY Stony Brook hired 9 new researchers.*

*SUNY Buffalo hired twenty-four graduate students, post-doctorates, and technicians.*

*At the University of Rochester, for example, an estimated eighty-three jobs have been created or maintained with state funding.*

*Weill Cornell Medical College filled nearly thirty new positions using a combination of state, federal and private funds.*

*The New York Stem Cell Foundation (“NYSCF”) hired eighteen new researchers with state funding and many more indirectly through the NYSCF’s own external grants and other funding sources.*

*Memorial Sloan Kettering Cancer Center (MSKCC)* has hired and maintained 27 full-time equivalents (FTEs). These are just a few of many examples.

### **Leverages Federal Funding and Private Philanthropy**

New York's state funding commitment also assists in leveraging federal funding. For example, New York state stem cell funding was instrumental in assisting Albert Einstein College of Medicine in obtaining \$10 million from the National Institutes of Health (NIH), issued under the American Recovery and Reinvestment Act (ARRA), to expand its stem cell research capabilities. The funds will be used to create new laboratories in order to increase its already substantial base of stem cell investigators.

The Mt. Sinai School of Medicine was also able to secure ARRA federal stimulus funding after receiving state funding for a shared facilities grant. In April 2010, the National Center for Research Resources, part of the National Institutes of Health (NIH), awarded nearly \$10 million to The Mount Sinai Medical Center to support the expansion, improvement, and consolidation of space for laboratories at Mount Sinai School of Medicine.

After SUNY Upstate obtained state funding to renovate laboratory space, they were able to leverage millions in NIH funding for additional research projects. Likewise, SUNY Buffalo was able to leverage NIH funding with the help of NYSTEM funds, and used data that was derived from NYSTEM funded work. The University of Rochester leveraged nearly \$2 million in NIH funding for training grants.

The State's stem cell investment also leverages institutional support. At SUNY Stony Brook, NYSTEM funding spurred institutional support for renovations, faculty costs and new hires.

The investment in stem cell research is also instrumental in leveraging funds from private philanthropy. For instance, the NYSCF raised \$70 million in philanthropy from independent foundations and individuals over a five year period.

### **Spurs Economic Development**

The total \$600 million commitment is enabling New York's medical schools and research laboratories to expand, and in some instances, create a stem cell infrastructure. Across the state, medical schools and research institutions are purchasing cutting-edge equipment, renovating laboratories and building state-of-the-art stem cell centers. The creation of these centers continues to spur economic development by keeping New York at the forefront of stem cell technologies and on the cutting edge of biomedical research.

In Upstate New York, the University of Rochester Medical Center (“URMC”) received \$3.3 million for the construction of a new facility that will enable scientists to produce human stem cells suitable for testing new therapies. This facility, the Upstate Stem Cell cGMP (“current good manufacturing practices”) facility, will be a regional resource available to scientists through Upstate New York and is expected to be completed by the end of 2011. A number of biotechnology companies have also formally expressed an interest in using the facility.

The University of Buffalo received \$3.5 million to establish a Western New York Stem Cell Culture and Analysis Center. Likewise, these investments are creating hundreds of new construction jobs.

At Weill Cornell Medical College, the Ansary Stem Cell Institute brings together a premier team of scientists to focus on stem cell research. The Ansary Institute takes a collaborative approach to stem cell research by bringing together scientists from varied areas in biomedical research. The Institute was established in 2004 with a generous gift from Mr. Hushang Ansary. Since then, the Institute has garnered approximately \$26

million in external funding, including support from the Starr Foundation Tri-Institutional Stem Cell Initiative and the Empire State Stem Cell Board (NYSTEM).

The NYSCF recently purchased equipment for small molecule high throughput screening and have established a lab that specializes in this type of research. Because of this, researchers from various New York institutions as well as out of state institutions have approached the NYSCF with the hope of collaborating with the Foundation on screen projects.

### **Stem Cell Cuts Would Create a Negative Ripple Effect on the Economy**

Last year's enacted budget reduced the \$50 million stem cell annual appropriation by \$5.2 million. This reduction compounded reduces the remaining 9 year commitment level by a total of \$47 million, bringing the original \$600 million commitment down to \$553 million. Although the State is facing an upcoming deficit, any further reduction to New York's stem cell program will have a significant negative ripple effect on the State's stem cell community as well as the State's economy. The dollars that New York's stem cell program sends out into communities, known as "extramural funding," provide real, direct economic benefits at the local level, including increased employment, growth opportunities for universities, medical centers, and local companies; and additional economic stimulus for the community. And when state funding is cut, communities across the state suffer too.

There is significant risk that researchers in New York will be recruited away to institutions in other states where they would have access to more resources to pursue their research, such as California, Connecticut, New Jersey, Illinois or Maryland. The loss of these scientists will have a negative effect on the State's entire research community as research grants, junior scientists, biotech companies, and venture capital will similarly migrate to those states that are perceived to be on the cutting edge of biomedical research. As we speak, institutions in other states – and even other countries – are

attempting to lure away New York's top scientists with the promise of public funding and a more welcoming regulatory climate for stem cell research. Any further reduction or elimination to the state's stem cell program will only worsen the "brain drain." The resulting loss of research talent would have a devastating ripple effect on New York's entire research population. New York could not only lose some of its top scientists to institutions in other states, but it will also become more difficult to recruit talented junior scientists who will see more opportunities to advance their research and careers in institutions that are pursuing cutting edge biomedical research.

In addition to lost jobs, New York's medical schools and research institutions would be forced to end or significantly reduce existing research projects. A number of research projects would come to a halt, and without other sources of support, would be in jeopardy of ending. As such, this would slow the progress in developing therapies that have the potential to drastically change the treatment of conditions like Alzheimer's disease, amyotrophic lateral sclerosis (i.e. ALS or Lou Gehrig's disease), burns, cancers, spinal cord injury, Parkinson's disease, juvenile diabetes and other conditions. Moreover, the new research facilities recently constructed would merely become "empty shells."

In addition to the academic and commercial impact of stem cell research, as the research advances to the stage of successful therapies New York could stand to realize billions of dollars in reduced health care costs. In New York, over \$94 billion is spent every year on health care – with taxpayers paying \$35 billion on that total.<sup>1</sup> A significant portion of that cost is for treating individuals with chronic conditions, many of which could benefit from stem cell research. In total, these diseases account for 73 percent of deaths in New York and account for 70 percent of total medical costs. Given the potential of stem cell research, New York could realize significant cost savings in future years.

As illustrated, it is essential to preserve New York's commitment in stem cell research in order for the State to continue to be one of the strongest research communities in the world.

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<sup>1</sup> See A Scientific, Policy and Economic Analysis: New York and Stem Cell Research. University of Rochester.

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New York’s medical schools, the New York Stem Cell Foundation (NYSCF) and Memorial Sloan Kettering Cancer Center (MSKCC) have received over 55 percent, or \$158 million, in total funding stem cell funding to date.

### Upstate New York

In Upstate New York, medical schools have been awarded \$35 million in stem cell funding to date. Over 100 jobs have been created.

Albany Medical College	2 awards	\$1.32 million
SUNY Buffalo	11 awards	\$10.0 million
SUNY Upstate	3 awards	\$6.4 million
University of Rochester	22 awards	\$18 million
<b>Total Upstate</b>	<b>38 awards</b>	<b>\$35.8 million</b>

### Downstate New York

In Downstate New York, medical schools, the New York Stem Cell Foundation and Memorial Sloan Kettering have been awarded \$123 million in funding to date. Nearly 200 jobs have been created.

Albert Einstein College of Medicine	13 awards	\$15.2 million
Columbia University Medical Center	21 awards	\$31 million
Mount Sinai School of Medicine	24 awards	\$23.1 million
New York Medical College	2 awards	\$300,000
NYU School of Medicine	11 awards	\$12 million
SUNY Stony Brook	9 awards	\$9.7 million
SUNY Downstate Medical Center	2 awards	\$312,000
Weill Cornell Medical College	8 awards	\$10.1 million
New York Stem Cell Foundation	4 awards	\$7.8 million
Memorial Sloan-Kettering	16 awards	\$13.2 million
<b>Total Downstate</b>	<b>109 awards</b>	<b>\$123 million</b>

### Other Award Recipients

Other award recipients include:

Cold Spring Harbor Laboratory	5 awards	\$2.3 million
Rensselaer Polytechnic Institute	4 awards	\$4.8 million
Regenerative Research Foundation	7 awards	\$4.8 million

Ordway Research Institute	3 awards	\$1.2 million
Cornell University	10 awards	\$6.5 million
Columbia University	12 awards	\$7.4 million
Wadsworth Center	2 awards	\$1.28 million
The Rockefeller University	2 awards	\$2.1 million
The NYS Psychiatric Institute	2 awards	\$1.6 million
Research Foundation for Mental Hygiene, Inc.	1 award	\$1.07 million
The Research Foundation of SUNY – SUNY Buffalo	1 award	\$1 million
The Research Foundation of SUNY – SUNY Stony Brook	1 award	\$1 million
Barnard College	1 award	\$227,452
Trudeau Institute	1 award	\$101,000
Brookhaven National Laboratory	1 award	\$76,800
City College of New York/CUNY	1 award	\$198,000
Syracuse University	1 award	\$324,000
The New School	1 award	\$212,000

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*The Associated Medical Schools of New York (AMSNY) is a consortium of the 16 public and private medical schools throughout New York State. The AMSNY mission is to support quality health care in New York State through the continual strengthening of medical education, medical care, and medical research. The combined total of New York's medical schools economic impact equals more than \$85.6 billion. This means \$1 in every \$13 in the New York economy is related to AMSNY medical schools and their primary hospital affiliates. For more information on AMSNY, please visit [www.amsny.org](http://www.amsny.org).*