

Empire State Stem Cell Board
STRATEGIC PLAN



NYSTEM

NEW YORK STATE
STEM CELL SCIENCE

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Message from the Chair of the Strategic Plan Coordinating Committee

In the spring of 2007, the New York State Legislature and the Governor committed \$600 million over 11 years to be spent on stem cell research. Their intent was to sponsor a strong research community in New York State that could explore the potential of stem cell technology to alleviate disease and improve human health. The legislation also created the Empire State Stem Cell Board, which in turn has prepared this Strategic Plan. The Plan is intended to guide the optimal use of the funds in order to accelerate scientific knowledge about stem cell biology and maximize the benefits of this investment for the residents of New York State.

Informed by the experience of other states, our intent is to form a public-private partnership between the State and its research and biotechnology communities. We believe this requires the Board to chart a careful course between the desire to spend the funds to find effective medical therapies as quickly as possible and the realization that there is much to learn about how stem cells function. Thus, the Plan proposes mission-directed initiatives for preclinical and clinical research to develop new therapies for human diseases. We also recognize that major scientific breakthroughs often result from basic research that is not necessarily targeted at specific diseases and, thus, have sought to maintain flexibility by proposing a research agenda that allows room for New York scientists to pursue their most innovative research ideas.

In developing this Strategic Plan, the Board engaged in numerous intense discussions on issues that did not have clear-cut solutions. The Board considered how to best ensure public access to the knowledge created by New York stem cell research and how to bring research findings to market while protecting the State's investment. We believe this requires a balance between protecting the interest of the public and the State and providing sufficient incentives to effectively bring complex technologies to the public. The Board will continue to explore this issue as it develops policies for intellectual property, technology transfer, and related topics.

The Board is grateful to the many individuals who gave generously of their time and energy over the course of many months to develop a thoughtful and comprehensive Strategic Plan that will guide the decisions of the Board for the next five years. The details of the planning process, as well as a list of members of the Strategic Plan Coordinating Committee and others who contributed to the Plan, are included in Appendix 2 and the Acknowledgements section.

Finally, just as we are not certain where the research envisioned here will take us, we know that this Strategic Plan must evolve in ways we cannot yet predict. The Board expects to revisit the Strategic Plan periodically as scientific progress gives rise to new opportunities and new challenges in stem cell research. We do believe it is important, though, to get started so that the investment made in stem cell research by the people of New York State can be fully realized and so that people everywhere who are living with diseases or injuries that might be alleviated by stem cell-based treatments can reap real benefits from this promising research.



Michael A. Stocker, M.D., M.P.H.

Chair, Strategic Plan Coordinating Committee

May 2008

EXECUTIVE SUMMARY

MISSION STATEMENT OF THE EMPIRE STATE STEM CELL BOARD

To foster a strong stem cell research community in New York State and to accelerate the growth of scientific knowledge about stem cell biology and the development of therapies and diagnostic methods under the highest ethical, scientific, and medical standards for the purpose of alleviating disease and improving human health.

Stem Cell Research: Progress and Promise

Stem cell research has captured the attention of the general public and scientists alike because of its potential to transform the treatment of countless human diseases. If researchers can develop safe, reliable methods to turn stem cells into a source of replacement cells for diseased or damaged tissues, millions of Americans living with devastating diseases or injuries could benefit. Indeed, stem cell therapy is already in use for some cancer patients who receive bone marrow or umbilical cord blood transplants, as well as for burn victims treated with stem-cell generated skin grafts.

Stem cells can be derived from a variety of sources, primarily embryonic and adult tissues. Human embryonic stem cells, which in theory could be directed to become any of the 200 or so cell types of the body, were first isolated and grown in a laboratory in 1998. This landmark research advance gave new hope to the possibility that currently incurable conditions, such as amyotrophic lateral sclerosis, type 1 diabetes, spinal cord injury, Parkinson's disease, and many others, could be treated and possibly reversed with stem cell-based regenerative medicine. The ongoing discovery of stem cells in adult tissues, such as the liver, brain, and fat, has provided another potential source of replacement cells, if researchers can find ways to harness their therapeutic potential. Notwithstanding the substantial progress made over the past decade, important challenges remain before the full potential of either embryonic or adult stem cell research is realized by individuals living with disease or injury. In parallel with scientific progress, stem cell research has raised important ethical, legal, and social issues that call for an open dialogue with the research community and the public. Specifically, the use of human embryos in stem cell research generates unique concerns that go beyond the general expectations of public accountability, respect for research participants, and scientific integrity that are common to all biomedical research disciplines. The Empire State Stem Cell Board (hereafter referred to as the "Board") is committed to an open and transparent forum for debating the moral and ethical issues related to such research. The statute establishing the Board specifically prohibits support for research that directly or indirectly involves human reproductive cloning.

NYSTEM: New York State Investment in Stem Cell Research

As the location of many world-class biomedical research organizations, New York is well-positioned to be a leader in the field of stem cell research and policy. In 2007, New York joined a growing number of States that are investing public funds in the support of stem cell research. The 2007-2008 enacted budget created the Empire State Stem Cell Trust Fund with a commitment of \$600 million in state funds over 11 years. In parallel, the Board was established and charged with making grants for basic, applied, translational, and other research and development activities that will advance stem cell research in New York State. The Board functions through two standing committees. The Funding Committee oversees the solicitation, review, and award of research grants supported by the Trust Fund. The Ethics Committee is responsible for making recommendations to the Funding Committee with respect to scientific, medical, and ethical standards related to stem cell research in New York State. The Trust Fund is administered by the New York State Department of Health through the New York State Stem Cell Science/NYSTEM program under the direction of the Board.

A Strategic Framework for Stem Cell Research Support in New York State

This Strategic Plan sets forth the Board’s vision for the use of the Trust Fund over the next five years. To maintain its ability to capitalize on breakthrough discoveries in the rapidly evolving stem cell research field, the Board views this Plan as a “living” document that will be re-assessed each year as a rolling five year plan. The Board has defined five overarching categories to organize the goals and expenditures of the Trust Fund. Within this framework, a projected range of funding support has been allocated to each category (see Table). As the Board reviews progress and funding priorities each year, the target distribution of the Trust Fund will be re-evaluated to ensure that the NYSTEM program responds to progress in stem cell science and continues to support the overall mission of the Board. For each category, the Board has formulated a mission statement to describe how expenditures in that category will contribute to the achievement of its overall mission. In addition, the Board has articulated specific goals to guide development of NYSTEM funding initiatives and other activities that will support the stem cell research enterprise in New York State.

Categories and Target Distribution of the Empire State Stem Cell Trust Fund

Category	Percent	Target Plan Expenditures (5-year)
Research	65-80%	\$195,000,000 – 240,000,000
Scientific Training	4-10%	\$12,000,000 – 30,000,000
Infrastructure Development	10-15%	\$30,000,000 – 45,000,000
ELSIE	3-5%	\$9,000,000 – 15,000,000
Administration	3-5%	\$9,000,000 – 15,000,000
TOTAL	100%	\$300,000,000

Research

Mission: Support innovative basic, translational, and clinical research that builds on the potential of stem cells to detect, treat, and cure human diseases.

The Board will invest the majority of the Trust Fund in the direct support of stem cell research and development. Research funds will be used for investigator-initiated projects that propose innovative directions in stem cell research, as well as targeted projects designed to capitalize on emerging opportunities. The goals of the program include understanding basic stem cell biology; deriving and characterizing new human pluripotent stem cell lines, including disease-specific lines; using those new lines to understand disease mechanisms and develop new therapeutic strategies; testing promising new therapies and diagnostic methods in preclinical models and early phase clinical trials; and developing mechanisms to support research collaboration within New York State and between New York researchers and their colleagues located elsewhere.

Scientific Training

Mission: Ensure a robust, interactive stem cell research community in New York State by providing training opportunities to support the entry of new and established investigators into stem cell research.

The Board will devote substantial, directed resources to training programs for stem cell scientists at all career stages including students and fellows in training; newly independent investigators who are launching careers in stem cell research; and established investigators with expertise that could be applied to stem cell research. In addition, conferences and workshops will be developed to foster communication, collaboration, and synergy within the New York State stem cell research community.

Infrastructure Development

Mission: Expand stem cell research capacity in New York State by establishing and ensuring access to appropriate infrastructure and resources.

The Board has allocated funds for institutions to develop infrastructure that will allow all interested New York State researchers access to critical resources for stem cell research. State-of-the-art facilities within institutions or at multi-institutional centers may be established to provide bioproduction or high-throughput screening capabilities, specialized equipment, technical support, and other resources for cutting-edge stem cell research. Resources for administrative support and regulatory guidance will foster translation of basic discoveries into clinical applications.

Ethical, Legal, and Social Issues and Education (ELSIE)

Mission: Ensure that stem cell research in New York State adheres to the highest standards of medical ethics and that the ethical, legal, social, and psychological implications of advances in stem cell research are appropriately addressed by engaging diverse communities in research, scholarship, and education on these issues.

The Board recognizes the need to increase understanding of fundamental ethical, legal, and social issues related to stem cell research. These issues will be addressed by open dialogue within and between the Ethics Committee and the Funding Committee of the Board, the public at large, and other private and public agencies, including other state governments, with an interest in stem cell research policies. Funds will be used to support research and to engage diverse communities within New York State in ways that enhance understanding of stem cell research, the ethical, legal, and social issues affecting this research field, and the impact of this research on society.

Administration

Mission: Manage the Empire State Stem Cell Trust Fund under the highest standards of accountability and integrity on behalf of the people of New York State.

Administrative functions, such as instituting a rigorous peer review process, program development and evaluation, and implementation of contract processes, will be carried out by the New York State Department of Health's Wadsworth Center in a manner that engenders public trust in the NYSTEM program. The Board is committed to ensuring public access to information regarding its activities and programs and to the outcomes of NYSTEM-funded research, training, and development.

Benefits of Promoting Stem Cell Research in New York, Evaluation, and Public Accountability

Mission: Ensure New York State preeminence in the application of knowledge derived from stem cell research for the greater public good and the generation of long-term support for stem cell research.

The evaluation of the NYSTEM program and ensuring public accountability are integral functions of this initiative. The Board is required to report annually to the public on its activities, grants awarded, grants in progress, research accomplishments, and future directions. Moreover, the Board will develop intellectual property, technology transfer, and fiscal policies to assure broad access to the results of NYSTEM-funded research. Importantly, the economic and other benefits derived from the State's investment in stem cell research will be assessed at regular intervals to maximize support for funding stem cell science and to use state funds to leverage additional commitments to stem cell research in New York. These activities will enable the Board to make informed decisions about program priorities and directions, and ensure that the promise of stem cell research is realized by the people of New York State.

I. Introduction

MISSION STATEMENT OF THE EMPIRE STATE STEM CELL BOARD

To foster a strong stem cell research community in New York State and to accelerate the growth of scientific knowledge about stem cell biology and the development of therapies and diagnostic methods under the highest ethical, scientific, and medical standards for the purpose of alleviating disease and improving human health.

Stem cell research is the foundation of regenerative medicine, which aims to improve human health and alleviate disease by restoring cells, tissues, or organs lost to disease or injury. For decades, doctors have harnessed stem cells found in bone marrow or umbilical cord blood to rebuild a patient's entire blood supply and immune system. Bone marrow or cord blood transplantation, as this technique is known, has proven to be a powerful treatment for diseases such as leukemia, lymphoma, sickle cell anemia, and others. Likewise, stem cells taken from healthy skin are routinely used to regenerate new skin grafts for burn patients. Although life-saving for many patients, these remarkable therapies address only a small number of the diseases and conditions that potentially could be treated with stem cell-based medicine.

The promise of stem cell research and regenerative medicine was boosted in 1998 when researchers first announced that they had isolated human embryonic stem cells (hESC) and successfully grown them in the lab. In theory, such cells can be directed to turn—or “differentiate”—into any type of cell in the human body. Using adult tissues, researchers have also isolated more specialized stem cells, such as those that give rise only to muscle cells or neurons. Scientists around the world are working to unlock the secrets of stem cell biology and find reliable ways to turn both embryonic and adult stem cells into replacement tissues and organs. In another important application, stem cells are being used to develop novel assays in the search for therapeutic drugs to counter chronic, degenerative diseases and to improve cancer therapies. However, significant research challenges remain before the potential of stem cell therapy can be fully realized. By establishing the Empire State Stem Cell Trust Fund (Trust Fund), the State of New York has signaled its long-term commitment to supporting stem cell research within the State and to accelerating the development of stem cell-based cures for human diseases for the benefit of all people throughout New York, the nation, and the world.

A stem cell is a single cell that can replicate itself or differentiate into many cell types.

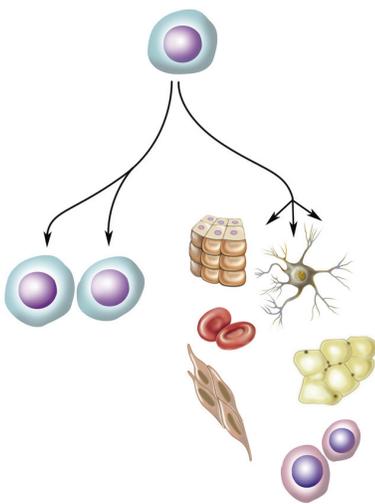


Image prepared by Catherine Twomey for the National Academies, *Understanding Stem Cells: An Overview of the Science and Issues* from the National Academies, <http://www.nationalacademies.org/stemcells>. Academic noncommercial use is permitted.

Stem Cells: An Overview

Stem cells have two defining properties: (1) self-renewal—that is, stem cells can maintain themselves in an undifferentiated state through multiple cycles of cell division; and (2) potency—when given the proper molecular cues, stem cells can produce differentiated cells with specialized functions. Stem cells found at different stages of development exhibit varying levels of potency. Totipotent stem cells are produced in the first few cell divisions after fertilization of an egg by a sperm; these unique cells eventually give rise to all cells of the embryo in addition to tissues needed for fetal growth, such as the placenta. As the cells of the early embryo (known as a “morula” at this stage) continue to divide, they form a blastocyst—a hollow sphere of cells surrounding a group of about 30 additional cells known as the inner cell mass. Each cell of the inner cell mass is pluripotent or capable of differentiating into all of the more than 200 types of cells found in the body. Finally, as the embryo matures into a fully formed organism, many organs retain stem cells

that are multipotent. Such cells can develop into a limited number of cell types and are used by the body throughout life to replenish and repair some tissues and organs.

Isolating pluripotent human stem cells has been a major challenge in biomedical research. Pluripotent hESC lines are derived from blastocysts that have been created by in vitro fertilization (IVF) techniques. To create these lines, stem cells are removed from the inner cell mass and grown in nutrients that allow the cells to remain in an undifferentiated state while replicating into larger numbers. Researchers are also exploring alternate methods of producing pluripotent human stem cells that do not entail the destruction of IVF-created blastocysts. One such method, called “somatic cell nuclear transfer (SCNT),” involves taking the nucleus from a differentiated adult or “somatic” cell, such as a skin cell, and inserting it into a donated human egg from which the original nucleus has been extracted. The egg, which now carries the genetic material of the adult cell, is then stimulated to form a blastocyst from which pluripotent inner cell mass cells can be harvested. “Altered nuclear transfer (ANT)” has been proposed as an alternative technique to create pluripotent stem cells. In ANT, either the somatic nucleus or the enucleated egg is modified such that the fused cell can give rise only to the pluripotent inner cell mass cell. In 2007, researchers reported a breakthrough by inducing human pluripotent stem cells using another new technique. “Induced pluripotent stem cells (iPS)” are created by using viruses to insert stem cell-associated genes into differentiated adult cells. Through pathways that are not yet understood, these stem cell genes appear to reprogram the adult cells back into an undifferentiated, pluripotent state.

Multipotent stem cells have been identified in several adult tissues, including bone marrow, umbilical cord blood, skin, liver, skeletal muscle, brain, and fat tissue. Although adult stem cells are normally capable of differentiating into only a few specific cell types within their tissue of origin, researchers are looking for ways to coax them into a wider variety of cell fates. If successful, such research could generate new sources of pluripotent stem cells or, at least, expand the therapeutic potential of multipotent cells. Importantly, adult stem cells have not yet been found in many tissues.

The Promise of Stem Cell Research for Treating Disease

Stem cell biology is relevant to almost all aspects of biomedicine and disease, including developmental abnormalities, degenerative diseases, immune dysfunction, and cancer. Millions of Americans live with diseases or injuries that might benefit from stem cell research—Alzheimer’s disease, amyotrophic lateral sclerosis (i.e., ALS or Lou Gehrig’s disease), burns, cancer, cardiovascular disease, type 1 (juvenile) diabetes, multiple sclerosis, osteoporosis, Parkinson’s disease, liver disease, sickle cell disease, spinal cord injury, stroke, and vision loss, to name just a few. The idea that diseased or damaged cells can be replaced with transplantable cells cultured in a lab is appealing. With the rapid growth in understanding stem cell biology, the potential uses of both embryonic and adult stem cells in tissue repair and cell-based therapies are being widely investigated. Already, encouraging results from animal models demonstrate the use of embryonic stem cells to reverse disease in liver failure, diabetes, sickle cell disease, and spinal cord injuries, among others. Clinical trials using hESCs, for example for the repair of spinal cord injuries, are being developed but have not enrolled human patients to date. Several therapies using adult stem cells are currently being tested in early phase clinical trials. For example, a trial is in progress to evaluate the safety of a stem cell-based treatment for children with Batten disease, an inherited brain disease.

Derivation of Stem Cells from Embryos

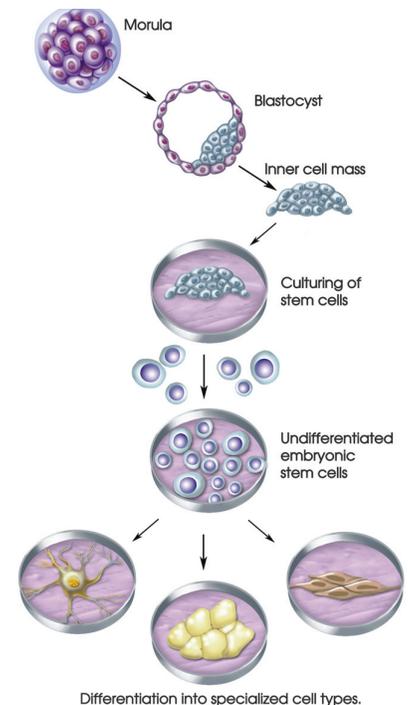


Image prepared by Catherine Twomey for the National Academies, *Understanding Stem Cells: An Overview of the Science and Issues* from the National Academies, <http://www.nationalacademies.org/stemcells>. Academic noncommercial use is permitted.

In addition to the development of cell-based therapies, stem cells can be used to study the nature of certain human diseases at the cellular level in ways that were not previously possible. For example, stem cells derived from patients with a particular disorder, such as ALS, can be used as tools to understand the pathophysiologic basis of the disease and to search for novel drugs that protect against or reverse disease. Stem cells also provide new ways to measure drug toxicities and to understand the effect of environmental insults and injury during development. Likewise, greater understanding of basic stem cell functions may allow researchers to manipulate these cells in their natural locations within the body. For example, research on stem cell renewal and differentiation could point to strategies for activating endogenous stem cells in order to repopulate or repair damaged tissues and organs without the need for transplantation.

Finally, stem cells may give rise to cancers, with major implications for cancer therapies. Many scientists now believe that normal adult stem cells can accumulate genetic mutations that trigger transformation into cancer stem cells or that more differentiated cancer cells acquire stem cell-like properties. In such models, these abnormal stem cells constitute only a small fraction of the cells in a tumor but are responsible for initiating tumors, fueling uncontrolled growth, giving rise to all of the different types of cells that are found in tumors, and seeding the spread of cancer to other sites. Moreover, cancer stem cells seem to survive drugs and radiation used for cancer therapies better than other tumor cells, perhaps by hiding in special stem cell “niches,” and may be responsible for cancer relapse. On the other hand, traditional cancer therapies may destroy normal stem cell populations in non-cancerous tissues, leading to devastating side effects. Given these considerations, research to better understand the relationships between stem cells and cancers should enable improved treatments and greater protection from the undesired effects of therapies.

Ethical, Legal, Social Issues and Education in Stem Cell Research

Stem cell research raises ethical, legal, and social issues that have garnered much discussion among the research community and the public. Many of these issues are unique to stem cell research or have unique aspects, including: the appropriateness of using human embryos in stem cell research; proper methods of obtaining informed consent for donation of human embryos or gametes (i.e., eggs); the creation of chimeras that combine human and non-human cells; the establishment of institutional oversight of human pluripotent stem cell research; and others. Understanding and addressing these issues require ongoing discussion among researchers, ethicists, health care providers, patients, advocacy groups, policy makers, and the general public. Stem cell research, like all biomedical research, is expected to conform to the highest possible standards of public accountability, respect for research participants, and scientific integrity. The Board is committed to an open and transparent forum for debating the moral, ethical, and other issues related to such research.

New York State’s Interest in Stem Cell Research

The National Institutes of Health (NIH), the nation’s largest funder of biomedical research, expends substantial funding on stem cell research, including studies of embryonic and adult stem cells from both animals and humans. However, NIH support for research on pluripotent hESCs—considered by many scientists to be the most promising starting point for cell-based therapies for a wide variety of diseases—is restricted by federal policies to a small number of approved hESC lines that were in existence before August 9, 2001. These approved lines have been

used by researchers across the country to generate important insights into stem cell biology. However, over time the quality of these hESC lines has begun to degrade, with many lines showing evidence of genetic instability. Moreover, the approved lines are all grown in the presence of mouse-derived “feeder” cells that provide important nutrients but which render the lines unsuitable for therapeutic applications in human patients. Finally, the few federally approved hESC lines do not adequately represent the genetic diversity of the national or global population. Any cell-based therapies derived from these lines would be rejected by the immune systems of many patients or would require co-administration of drugs to suppress the immune system. These concerns highlight the need for making additional pluripotent stem cell lines available for research.

Given the rapid pace of scientific discoveries in the stem cell field, federal policies have left a significant gap in funding for research on more recently derived, robust hESC lines that can be grown in the absence of animal cells. Moreover, the NIH cannot support the derivation of new hESC lines using up-to-date techniques or from blastocysts with disease-specific characteristics or lines that are genetically and immunologically matched to individual patients. The federal policies have created an opportunity for state governments to fill this critical funding gap and, in the process, foster vigorous stem cell research communities in their states. As of April 2008, California, Connecticut, New Jersey, Illinois, and Maryland are among the states that have authorized the use of public funds for stem cell research.

New York is well-positioned to be a leader in the field of stem cell research because of its wealth of world-class research universities and institutions, teaching hospitals, and biotechnology and pharmaceutical companies. Biomedical research and biotechnology already have significant economic impact in New York, with estimates of over 500,000 jobs and nearly \$50 billion in economic activity.¹ Stem cell biology links many aspects of biomedicine, and developments in this field will stimulate research and discovery in related fields of biomedical science. Support of stem cell research in New York State will help prevent the loss of leading research scientists to other states and countries in the current, highly competitive environment and provide an opportunity to leverage other state and federal government investments in the New York academic research and biotechnology enterprises. Importantly, the New York stem cell research program will take place in the context of ongoing public discourse, ethical analysis, and oversight to ensure that the research will be conducted with the highest regard for scientific and medical ethics.

The Empire State Stem Cell Trust Fund and the Empire State Stem Cell Board

The State of New York has committed to invest \$600 million in stem cell research over 11 years. The 2007-08 enacted budget created a Special Revenue Fund known as the Empire State Stem Cell Trust Fund. An initial appropriation of \$100 million in funding was provided in state fiscal year 2007-08, and another \$50 million will be transferred to the Trust Fund each year for the subsequent 10 years.

The Empire State Stem Cell Board was established within the Department of Health for the purpose of administering the Trust Fund. The Board (see Appendix 1) is empowered to make grants for basic, applied, translational, or other research and development activities that will advance scientific discoveries in fields related to stem cell biology. The Board functions through two standing committees as defined by the Public Health Law. The Funding Committee oversees the solicitation, review, and award of research grants supported by the Trust Fund. The Ethics Committee

¹New York and Stem Cell Research: a Scientific, Policy and Economic Analysis
<http://www.rochester.edu/news/pdfs/stemcellwhitepaper.pdf>

is charged with making recommendations to the Funding Committee with respect to scientific, medical, and ethical standards related to stem cell research. As part of this function, the Ethics Committee provides an accessible and transparent forum for exploration and discussion of ethical, legal, and social issues related to stem cell research in New York and the nation. Each year, the Board must report to the public on its activities, grants awarded, grants in progress, research accomplishments, and future directions of the program. The Department of Health’s Wadsworth Center has been given the responsibility for assisting the Board in carrying out its functions through the program called New York Stem State Cell Science/NYSTEM.

Framework of a Strategic Plan for Stem Cell Research in New York

The Empire State Stem Cell Trust Fund provides up to \$600 million over 11 years for stem cell research in New York State. To maintain flexibility and allow the Board to respond quickly to new breakthroughs in stem cell research, this Strategic Plan (see Appendix 2 for a description of the strategic planning process) accounts for \$300 million in expenditures over 5 years, with the expectation that the Plan will be re-assessed on an annual basis as a rolling five year plan. It is anticipated that an equivalent amount (\$300 million) will be available in years 6-11.

The Board has defined five overarching categories to organize the goals and projected expenditures of the Trust Fund: Research; Scientific Training; Infrastructure Development; Ethical, Legal, and Social Issues and Education (ELSIE); and Administration. Within this framework, the Board has projected a range of funding support to be allocated to each category (Table 1). The Board will conduct annual reviews to assess program progress and directions, and ensure that funding priorities continue to be consistent with the state of stem cell science and responsive to the overall mission of the Board. In the course of these reviews, the Board may choose to adjust the target expenditures. Actual expenditures and distribution of funds will depend on the quality of applications received and the availability of funds each year.

**Table 1:
Categories and Target Distribution
of the Empire State Stem Cell Trust Fund**

Category	Percent	Target Plan Expenditures (5-year)
Research	65-80%	\$195,000,000 – 240,000,000
Scientific Training	4-10%	\$12,000,000 – 30,000,000
Infrastructure Development	10-15%	\$30,000,000 – 45,000,000
ELSIE	3-5%	\$9,000,000 – 15,000,000
Administration	3-5%	\$9,000,000 – 15,000,000
TOTAL	100%	\$300,000,000

The categories for expenditure of the Empire State Stem Cell Trust Fund are:

Research: The Board will invest the majority of the Trust Fund in the direct support of stem cell research and development. Limited federal support for innovative research on pluripotent stem cells and a highly competitive State funding environment make

it essential to foster pioneering approaches in stem cell research. New York's program will enable scientists working in relevant areas to pursue research on key questions that must be addressed before fundamental stem cell research can be translated into new therapies. Research funds will support investigator-initiated research projects that propose innovative directions in stem cell research, as well as projects designed to capitalize on emerging opportunities in stem cell research.

Scientific Training: Stem cell science is a relatively new and rapidly expanding field, and trained personnel are in short supply at all levels of the research enterprise. The Board will devote substantial directed resources to training programs that will attract undergraduate students who are interested in pursuing a research career in stem cell biology, scientists at the early stages of their careers in stem cell research, and more experienced researchers who could apply stem cell research techniques to expand the focus of their work. These diverse training programs, along with events to foster communication among stem cell researchers in New York and elsewhere, will cultivate a robust community of stem cell researchers in the State.

Infrastructure Development: To foster a strong stem cell research community, the Board has allocated funding for institutions to develop infrastructure that will allow all interested New York State researchers access to critical resources for stem cell research. Procurement of major equipment and establishment of shared core facilities will encourage efficiencies and promote the wider application of state-of-the-art technologies to stem cell research throughout the State.

Ethical, Legal, and Social Issues and Education (ELSIE): Funds will be used to increase understanding regarding scientific, ethical, legal, and social matters pertinent to stem cell research. In addition, the Board will support the development of Requests for Applications (RFAs) targeted at ongoing examination of ethical issues related to stem cell research, as well as the development of public outreach materials and events to educate a broad audience on the promise and outcomes of stem cell research.

Administration: Administrative functions, such as establishing a peer review process, program development, and program oversight, are essential to ensure the appropriate use of resources, public transparency, and ongoing evaluation of program activities. Costs for administration of the funding program have been kept to a minimum by housing the NYSTEM program within the New York State Department of Health's Wadsworth Center and using existing personnel and facilities to the extent possible.

Benefits of Promoting Stem Cell Research in New York, Evaluation, and Public Accountability

In developing its Strategic Plan, the Board intends to create a "living document" that guides funding towards important and productive goals for research, training, infrastructure development, and ethical direction while retaining flexibility to respond to emerging opportunities in stem cell research. Thus, the Board will undertake a yearly evaluation of progress in stem cell research and development achieved through NYSTEM funding as part of its annual report to the people of New York State. The Board's mandate for public accountability will also be addressed through assessment of the economic impact of the program in New York State. These scientific and economic evaluations will promote transparency, enable informed decision making, and maintain the public trust with respect to New York State's investment in stem cell research.

2. Advancing the science of stem cell biology in New York

MISSION

Support innovative basic, translational, and clinical research that builds on the potential of stem cells to detect, treat, and cure human diseases.

GOALS

- Increase the understanding of basic stem cell biology, including stem cell proliferation, differentiation, and maintenance.
- Support the derivation, screening, and characterization of novel human pluripotent stem cell lines, including disease-specific cell lines.
- Promote the use of novel stem cell lines to understand the pathogenesis of diseases and to translate discoveries into new therapeutic strategies.
- Accelerate the development of novel stem cell-based therapies and bioassays for drug development and test these new approaches in preclinical models.
- Translate basic research discoveries into new therapies and diagnostic methods for testing in early phase clinical trials.
- Promote collaboration and communication among New York State institutions and with researchers throughout the United States and internationally.

Introduction

Despite great progress in recent years, these are still early days in the medical application of stem cells to human disease problems. Many gaps remain in our understanding of the basic biology of both embryonic and adult stem cells. New means of generating pluripotent stem cells without destruction of IVF-derived embryos have been described, but it is not known if those cells fully recapitulate the characteristics of hESC cells. Studies to compare the properties and the differentiation potential of hESC to those of pluripotent stem cells from other sources are required in order to determine which cells are most useful for different lines of research or clinical applications. Clinical trials using embryonic stem cells are being planned for spinal cord injury and other conditions. New types of adult stem cells continue to be identified, but the full differentiation potential has not been defined for many of these cells. Early phase clinical trials are underway using adult stem cells for a range of conditions that includes traumatic brain injury, myocardial infarction, Crohn's disease, multiple sclerosis, and graft-versus-host disease. However, researchers are still working to understand the potential benefits, as well as possible side effects, of transplanting these cells into human patients. Thus, much fundamental research is needed to translate stem cell science into effective human therapies.

The primary focus of New York's stem cell research funding program is the support of innovative research that will advance our understanding of stem cell biology and translate basic research discoveries into new therapies and diagnostic methods for human diseases. Both investigator-initiated and targeted research projects using animal or human embryonic and adult stem cells will be funded, subject to Board standards for hESC research. By law, no grants supported by the Trust Fund will be awarded or utilized for research involving human reproductive cloning.

Significance of Goals for Advancing the Science of Stem Cell Biology

- *Increase the understanding of basic stem cell biology, including stem cell proliferation, differentiation, and maintenance.*

Development of cell-based therapies requires a better understanding of how to maintain “stemness” (properties that uniquely define stem cells) during expansion of the cells in culture. Conversely, researchers are also working on strategies to direct stem cell differentiation into desired cell types, such as insulin-producing cells of the pancreas that could be used to treat type 1 diabetes or dopamine-producing neurons for treatment of Parkinson's disease. Understanding the role of the microenvironment or “niche” in controlling whether a stem cell remains in a pluripotent state or sets off down a differentiation pathway represents another vital area of research. The list of fundamental research questions in stem cell biology will continue to grow as some questions are answered and others arise from future research advances.

Basic research is inherently unpredictable, and some of the most exciting breakthroughs have come from researchers who made unexpected observations. For this reason, the Board has placed a high value on supporting investigator-initiated research proposals that promote creativity and allow researchers to pursue their most interesting and novel hypotheses. In addition, the NYSTEM program will

have the flexibility to respond rapidly to paradigm-shifting discoveries that could accelerate progress on the strategic goals by supporting targeted research initiatives.

- *Support the derivation, screening, and characterization of novel human pluripotent stem cell lines, including disease-specific cell lines.*

Human embryonic stem cells can be created from IVF-derived embryos that have undergone pre-implantation genetic diagnosis and are known to carry genetic markers of specific diseases. Such disease-specific hESC lines have been reported for a range of disorders that includes fragile X syndrome, Duchenne muscular dystrophy, Huntington's disease, Fanconi anemia, and others. These lines serve as key model systems for research on the effects of disease-associated genetic mutations on cell growth and differentiation. In addition, the disease-specific cell lines might be useful for screening potential drugs to prevent or reverse the defects associated with the particular disease. More recently developed techniques, such as somatic cell nuclear transfer (SCNT) or reprogramming adult cells to revert to a pluripotent state (i.e., induced pluripotent stem cells [iPS]), open new possibilities for creating pluripotent stem cell lines that are not only disease-specific but are also genetically matched to an individual patient.

NYSTEM funding will be available to support fundamental research to further develop SCNT and iPS technologies; to characterize the resulting stem cells in relation to hESC; and to create disease-specific human pluripotent stem cell lines from a variety of sources and techniques, including donated IVF-derived embryos, SCNT, and reprogramming of somatic cells.

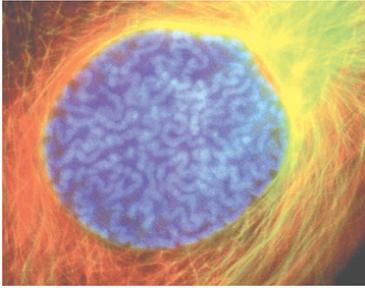
- *Promote the use of novel stem cell lines to understand the pathogenesis of diseases and to translate discoveries into new therapeutic strategies.*

The availability of disease-specific pluripotent lines will open up important new avenues of research on the causes and treatments for many human diseases. For example, researchers could reprogram a skin cell from a patient with ALS to generate a line of pluripotent stem cells that could then be used to investigate the pathogenesis of ALS or to develop bioassays to screen for potential new drugs to combat the disease. In the long term, if such research led to the development of a stem cell-based therapy for ALS, then cells from an iPS-derived line could be transplanted back into the original patient without risk of immune rejection. The NYSTEM program will support research that uses disease-specific stem cell lines developed in New York State or elsewhere to improve understanding of and develop therapies for human diseases.

- *Accelerate the development of novel stem cell-based therapies and bioassays for drug development and test these new approaches in preclinical models.*

Stem cell research opens up several potential avenues for therapeutic development. The most obvious application of this research is the differentiation of stem cells into specialized cells that can be transplanted to replace or repair diseased or injured tissues. For example, neural stem cells could be harnessed to restore function after a spinal cord injury. However, advances from stem cell research could also lead to approaches for stimulating stem cells in their natural locations in the body so that tissue repair can be achieved without the need for transplantation. Moreover, stem cells can be used for the development of bioassays to screen libraries of chemicals to identify those that might have therapeutic potential.

Therapies derived from any of these strategies must eventually be tested in preclinical animal models before researchers can conduct clinical trials with human patients. Preclinical studies are needed to assess the potential efficacy of stem cell-derived therapies and to address important safety considerations, including the possibility that transplanted stem cells could form tumors if the cells are not fully differentiated. New York will support translational research to rigorously explore the therapeutic applications of stem cells and to evaluate promising treatments in animal models.



- *Translate basic research discoveries into new therapies and diagnostic methods for testing in early phase clinical trials.*

Bone marrow or umbilical cord blood stem cell-based therapies, which have been used in clinical practice for decades, have revolutionized cancer chemotherapy. Therapies based on more recently discovered adult stem cells or embryonic stem cells are beginning to be developed and will require substantial, ongoing basic and translational research before new treatment strategies are ready to be tested in clinical trials. The funding program will support testing of highly promising, novel therapies in early phase clinical trials to establish proof-of-concept and safety in human patients. Later stage trials to assess efficacy of therapies in large numbers of patients over long periods of time will likely necessitate industry participation and investment.

- *Promote collaboration and communication among New York State institutions and with researchers throughout the United States and internationally.*

Progress in stem cell research can be accelerated by collaborations among researchers with complementary expertise, technologies, and resources who come together to form interdisciplinary teams, core facilities, and synergistic training and education programs. The development of multi-institutional, vertically-integrated teams of researchers from across the basic to clinical research spectrum is especially important to foster the development of bench-to-bedside approaches to therapeutic development for specific diseases. Partnerships between investigators in academic and other non-profit organizations and industry collaborators will be supported when appropriate. The allocation of funds for research collaborations and consortia will help establish New York as an attractive location for stem cell research, development, and commercialization.

Research Agenda

Given the rapid expansion of knowledge in stem cell science, it is not possible to predict where or when the next breakthroughs will occur. For that reason, the Board's research agenda is intended to be flexible and free of excessive constraints on the specific research topics that will be supported. Broad initiatives will be issued to solicit investigator-initiated research, including collaborative projects, that allow researchers to pursue their best, most promising scientific hypotheses related to stem cell research. However, the Board will also develop targeted initiatives that capitalize on recent advances in stem cell science and that address the specific goals set forth in this Strategic Plan. The timing of the release of RFAs and the receipt of applications in each funding year will be flexible.

Table 2 on the next page provides a list of funding initiatives that have been issued, are pending release, or are proposed for development as of May 2008.

**Table 2:
NYSTEM Funding Initiatives for Stem Cell Research**

Initiative	Release Date
Institutional Development for Stem Cell Capabilities	November 2007
Collaborative Research Planning Grants	Pending
Collaborative projects to focus on specific diseases or platforms	Proposed
Investigator-Initiated Innovative Research Grants	
- FY 2008	Pending
- FY 2009	Proposed
- FY 2010	Proposed
- FY 2011	Proposed
Targeted Research Grants	
- Investigation of iPS and other derivation approaches	Pending
- Early lineage development of stem cells	Proposed
- Stem cell niche/microenvironment interactions	Proposed
- Overcoming immunological barriers	Proposed
- Stem cells and cancer	Proposed
- Translational grants to advance disease-based projects into the preclinical phase	Proposed
- Early phase clinical trials	Proposed

Funding to Date

Institutional Development for Stem Cell Capabilities: In November 2007, the NYSTEM program was launched with an RFA for Institutional Development for Stem Cell Capabilities. One-year grants designed to increase the capacity of New York research institutions to engage in stem cell research were awarded in January 2008. Seventy-eight grants (\$6.1 million) were made to New York researchers to supplement or continue their active stem cell research projects. Additional funding was awarded to support stem cell research training programs (\$1 million, 23 awards) and infrastructure development (\$7.4 million, 41 awards) within New York State research entities.

3. Training stem cell researchers

MISSION

Ensure a robust, interactive stem cell research community in New York State by providing training opportunities to support the entry of new and established investigators into stem cell research.

GOALS

- Attract students and fellows in training to the field of stem cell research.
- Support new investigators in developing careers in stem cell research.
- Support cross-disciplinary training of established investigators with expertise that could be applied to stem cell research.
- Sponsor stem cell research conferences and workshops to promote information sharing and communication among New York State researchers and organizations, and their national and international counterparts.

Introduction

The field of stem cell research is in the early stages of discovery, particularly as it applies to human stem cells and the development of therapies for human diseases. Since the derivation of pluripotent hESC was first reported a decade ago, many important advances have been made in understanding stem cell biology and learning how to coax stem cells down specific differentiation pathways. However, continued progress in the stem cell research field as a whole depends on attracting the best and brightest researchers at all levels of training and career development to apply their scientific talents to stem cell research.

Federal policies on hESC research, which limit federal funding to a small number of approved hESC lines, have had the unintentional consequence of discouraging many new and established investigators alike from engaging in stem cell research. Thus, these policies create a critical need for the Board to support the development of programs designed to foster a cadre of stem cell researchers at all stages of the career pipeline. It is essential to provide opportunities that allow students and new investigators to pursue innovative stem cell research at a time when they are trying to launch productive research careers. Equally important are programs that would enable more established investigators to learn stem cell research techniques so that they can apply their unique skills, expertise, and technologies to research in this field. Lastly, conferences and workshops can promote communication, collaboration, and synergy within the New York State stem cell research community.

Significance of the Goals for Training Stem Cell Researchers

- *Attract students and fellows in training to the field of stem cell research.*

To ensure a future population of biomedical researchers and technical staff with the knowledge and desire to move the field forward, New York State must stimulate and support interest in stem cell-related biology at many points along the scientific education and training continuum. Short-term opportunities for undergraduates and medical students can introduce these young students to the challenges and promise of stem cell research at a time when they are beginning to make decisions about the direction and focus of their future careers. Longer fellowships provide an opportunity for graduate students, postdoctoral fellows, and clinical fellows to become fully immersed in stem cell research and to establish a foundation for future independent research careers in the field.

- *Support new investigators in developing careers in stem cell research.*

To sustain a strong and innovative stem cell research community, New York must be able to recruit and retain promising young stem cell investigators in the early stages of their careers. New investigators need access to stable funding, resources, and protected time for those with clinical responsibilities in order to pursue innovative research projects that can form the basis of a productive career. Early career development awards that offer an extended commitment are necessary to encourage promising new stem cell investigators to choose to develop their careers in New York. Similar awards could be developed for investigators who are more established to consider expanding into stem cell research.

- *Support cross-disciplinary training of established investigators with expertise that could be applied to stem cell research.*

Investigators working in such areas as biomedical imaging, tissue engineering, developmental biology, disease-oriented research, clinical research, and many other fields are all needed to move stem cells from the laboratory to clinical practice. To fully realize the potential of human embryonic stem cell research, it is critical to reduce the barriers to participation in this research. One such barrier is the difficulty in growing embryonic stem cells in culture. In addition, state-of-the-art techniques, such as reprogramming differentiated cells back into a pluripotent state, require intensive hands-on training for investigators who want to establish these protocols in their own laboratories. The development of courses to educate investigators on stem cell research techniques will help foster a robust community of stem cell researchers in New York State that has all of the tools and capabilities required for cutting-edge research and discovery in stem cell biology. Giving established investigators opportunities to acquire experience in stem cell research is another important mechanism for promoting participation in the field.

- *Sponsor stem cell research conferences and workshops to promote information sharing and communication among New York State researchers and organizations and their national and international counterparts.*

Scientific progress relies on regular communication among researchers who share new data, hypotheses, techniques, and technologies. Conferences and workshops are essential tools to promote communication among investigators working in the stem cell field, as well as between stem cell researchers and those from other, related fields who can contribute new perspectives to the challenges and problems in stem cell research. By bringing together stem cell researchers from organizations across New York State and their national and international colleagues, these events can help to stimulate collaboration among researchers within the State and between New York researchers and others in the broad stem cell research community.

4. Developing infrastructure for stem cell research

MISSION

Expand stem cell research capacity in New York State by establishing and ensuring access to appropriate infrastructure and resources.

GOALS

- Support state-of-the-art facilities and resources for stem cell research, including bioproduction facilities, equipment, and trained technical support.
- Establish multi-institutional stem cell research centers that support inter- and intra-institutional collaboration and serve as resource centers for New York stem cell researchers.
- Establish facilities for high-throughput chemical and genetic screening of stem cells and their derivatives.
- Develop resources for administrative support and regulatory guidance to assist basic stem cell researchers in translating their discoveries into the clinic.

Introduction

One of the challenges created by the federal policies on hESC research is that research entities must clearly separate resources and infrastructure used for research on approved hESC lines from those used for research projects involving stem cells that do not qualify for federal funding. Many institutions have responded to this requirement by establishing meticulous administrative and accounting processes to separate federal and non-federal funds for stem cell research or by constructing discrete facilities for non-federally supported research. Others have opted to allow their investigators to engage solely in stem cell research that is eligible for federal funding.

Through the Trust Fund, New York has an opportunity to catalyze pioneering research by establishing and ensuring statewide access to state-of-the-art infrastructure and resources for stem cell research. In addition to facilities for conducting research on hESC lines that are not on the federal stem cell registry, many investigators need access to specialized equipment for any type of stem cell research. Access to high-quality facilities and equipment will help to create and sustain a vigorous stem cell research effort in the State. Furthermore, the translation of basic science discoveries into clinical applications could be accelerated by the development of resources to guide investigators through the administrative and regulatory requirements of clinical development.

Significance of the Goals for Developing Infrastructure for Stem Cell Research

- *Support state-of-the-art facilities and resources for stem cell research, including bioproduction facilities, equipment, and trained technical support.*

Maintaining stem cells in culture in an undifferentiated state can be technically difficult and requires specialized equipment and trained personnel. In some institutions, duplicate facilities are maintained to separate research funded by federal and non-federal sources. In addition, testing stem cell-based therapies in clinical trials with human patients will require the establishment of bioproduction facilities that can produce stem cells under good manufacturing practice (GMP) conditions. Supporting the acquisition of state-of-the-art facilities, equipment, and resources within New York research organizations will help build capacity for stem cell research and development in the State and promote retention and recruitment of a strong, competitive stem cell research community.

- *Establish multi-institutional stem cell research centers that support inter- and intra-institutional collaboration and serve as resource centers for New York stem cell researchers.*

Multi-institutional research centers are essential to expand research capabilities to New York State investigators at institutions that do not have adequate facilities for stem cell research in general or for research on hESC lines that are not eligible for federal funding. Such shared facilities have the added benefits of fostering the efficient use of resources and promoting inter-institutional collaborations.

- *Establish facilities for high-throughput chemical and genetic screening of stem cells and their derivatives.*

The application of stem cells for drug development or bioassays requires access to highly specialized, costly equipment and reagents for high-throughput chemical and genetic screening that may include, but are not limited to: robotic liquid handling, automated microscopy, image acquisition software, small molecule libraries, and tools for gene over-expression or knock-out (e.g., lentiviral vectors). Establishing shared facilities for high-throughput screening of stem cell lines and their derivatives (e.g., proteins, supernatants) would improve the ability of New York stem cell researchers to characterize novel stem cell lines and to make use of those cells for therapeutic development.

- *Develop resources for administrative support and regulatory guidance to assist basic stem cell researchers in translating their discoveries into the clinic.*

Major hurdles in the development of stem cell-based cures for human diseases are the time-consuming, complex administrative and regulatory processes that must be followed before promising therapeutic ideas can be tested in human subjects. For basic scientists who have made breakthrough discoveries in stem cell science, having access to administrative support and regulatory guidance can make the difference between continuing to pursue fundamental research questions or translating their discoveries into clinical applications. The development of readily available resources to provide this guidance to New York State researchers is crucial for achieving clinical outcomes through the NYSTEM program.

5. Ethical, legal, and social issues and education (ELSIE) in stem cell research

MISSION

Ensure that stem cell research in New York State adheres to the highest standards of medical ethics and that the ethical, legal, social, and psychological implications of advances in stem cell research are appropriately addressed by engaging diverse communities in research, scholarship, and education on these issues.

GOALS

- Evaluate ethical, scientific, medical, legal, and social issues related to stem cell research and establish ethical standards for NYSTEM-funded research.
- Support research and scholarship on ELSIE issues as they relate to stem cell research that will help advance the research itself and inform public policy.
- Engage diverse communities in order to enhance public understanding of critical ethical, legal, and social issues and provide opportunities for education on stem cell research and its impact on society.

Introduction

New York State support for stem cell research can benefit citizens of New York, the nation, and the world by expanding our understanding of basic biological processes, by illuminating the complexity of human life, and, ultimately, by improving human health. Although stem cell research holds great promise for the development of therapies to treat human disease, such research must be sensitive to important ethical, legal, and social concerns and adhere to the highest scientific and ethical standards. The establishment of the Ethics Committee as an integral part of the NYSTEM program provides an opportunity for the State and the Board to take an active role in public education and discussion. Moreover, the Ethics Committee will enable the Board to respond to new and existing ethical challenges in stem cell research and to provide guidance in the development of public policies for conducting stem cell research under the highest standards of medical ethics.

The Board will take a synergistic, multi-pronged approach to study, discuss, and understand the complexity of the ethical, legal, and social issues raised by stem cell research and make recommendations. Some issues may best be addressed under the leadership of the Ethics Committee through deliberations and public dialogue. For certain matters, consultative input from recognized experts will be critical, as understanding of the science is necessary for establishing solid ethical parameters. Other questions may require funding initiatives that will support research and scholarship on various issues. In many instances, these efforts will be collaborative as the Funding Committee and the Ethics Committee confront new challenges and jointly guide the NYSTEM program. Flexibility is important in these complex areas, since it is impossible to predict all of the scientific developments that may occur or the ethical, legal, social, and educational implications that may arise. Often, it is not until researchers begin to conduct research that ethical questions and challenges emerge that then need to be addressed. For this reason, collaborative, open, and ongoing dialogue between the Ethics Committee and the Funding Committee will be important to generate perspectives that highlight common ground and acknowledge areas of disagreement among individual members and to jointly formulate policies that will guide research funded through the NYSTEM program.

Significance of Goals Related to Ethical, Legal, and Social Issues and Education in Stem Cell Research

- *Evaluate ethical, scientific, medical, legal, and social issues related to stem cell research and establish ethical standards for NYSTEM-funded research.*

The Ethics Committee of the Board is charged with making recommendations to the Funding Committee regarding scientific, medical, and ethical standards for stem cell research. The Funding Committee, in turn, has responsibility for adopting standards for stem cell research funded through the NYSTEM program. On an interim basis, the Funding Committee has decided that all human embryonic stem cell research funded by the Board must be reviewed and approved by an institutional Embryonic Stem Cell Research Oversight (ESCRO) committee and must comply with guidelines set forth by the National Academy of Sciences (NAS) or the International Society for Stem Cell Research (ISSCR), with some modifications.²

²The NYSTEM guidelines for stem cell research will be posted on the website.

The goals of this Strategic Plan as it applies to the ethical, legal, and social issues surrounding stem cell research will be addressed primarily through ongoing dialogue within the Ethics Committee, with external research and input from outside experts as needed. The Ethics Committee has identified multiple topics that will require further discussion in order to develop cogent recommendations regarding the standards for stem cell research in New York State. Although additional topics may be identified as research progresses, specific issues for examination include, but are not limited to:

Institutional oversight of stem cell research: The Board requires most human pluripotent stem cell research funded through the NYSTEM program to be reviewed and approved by ESCROs. These committees have been established at some institutions and will be needed at others. Since ESCROs serve a critical role in ensuring hESC research complies with the highest ethical, medical, and scientific standards, the Ethics Committee has reviewed the current composition, policies, and practices of ESCROs and recommended additional standards. The Ethics Committee is expected to conduct an ongoing review of ESCROs and consider the development of model policies, information, and educational materials that would help ensure these entities fulfill their intended purpose.

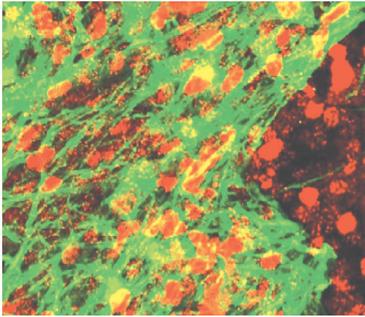
Ethical issues related to the source of human pluripotent stem cells: All medical practice, scientific research, and human subjects research should strive to treat people with respect. To explore and understand this value and how it is put into practice in stem cell research is clearly of great significance. The Board recognizes that there are strongly held, divergent views. Some individuals believe that human embryos possess a moral status that would preclude their use in any kind of medical research. Others do not accept that premise and, given the extraordinary promise of stem cell therapeutics, believe it could be considered unethical to block or even retard progress in this area of research in any of its forms. Many believe that embryos deserve special protection, and have distinct and special characteristics more than those held by other types of human cells but that if these issues are carefully considered, and if the intent of the research is to yield benefits for many patients suffering from disease, research involving human embryonic cells is ethically permissible. In a modern, pluralistic society with a wide diversity of viewpoints, full consensus on all of these issues is likely to be elusive. The Board will strive to ensure that sensitivity and respect on the part of scientists and others involved with stem cell research will be maintained. By law, NYSTEM funding cannot be used for research on human reproductive cloning.

Informed consent: Appropriate informed consent must be obtained from individuals participating in stem cell research, including those who provide oocytes and embryos, as for all areas of biomedical research. In the development of standards for funded stem cell research, the Board will consider key issues relevant to informed consent. These will include the level of comprehension among potential research participants concerning stem cell research and its discernible risks and potential benefits; how participants make decisions about possible participation; potential participants' understanding and preferences concerning future use and storage of biological or other genetic material; and how participants' concerns may be best addressed by researchers. Studies have explored ways to optimize the effectiveness of explanations about the complexities of stem cell research to increase the likelihood that participants in this research have given consent that is truly informed.

Payment for participation in research: Questions of whether gamete donors in stem cell research should be paid and, if so, how much, have been debated vocally and will

be addressed by the Board. Some research has been published on the possibility of different payment models for research participation in other areas of medicine (e.g., a wage model vs. a “compensation” model). However, the Board’s decisions regarding these issues may benefit from additional research on: the sociodemographic and other characteristics of women who choose to provide embryos or oocytes; how potential “donors,” patients, and others view payment for gametes; what amount of payment would be viewed as adequate; whether payment levels similar to those used in clinical situations, such as IVF, would be appropriate; and whether it is ethical to prohibit payment of donors.

Chimeras: The Board has required funded research to comply with the ISSCR or NAS guidelines which prohibit research aimed at the integration of human and non-human cells or genetic sequences (“chimeras”) in certain situations that pose unique risks. Yet, questions of which integration of human and non-human cells constitute areas of concern and which do not remain unresolved. For example, products of animal cells have already been incorporated into several human therapeutic interventions, including the use of pig valves for heart repair and of animal cell-derived hormone replacements. Hence, the appropriate definition of “unacceptable” chimeras will be addressed and developed.



Justice issues: State funding of innovative research raises critical questions regarding how the benefits of such research should be distributed to citizens and whether residents will have access to new therapies and diagnostic methods developed with state funds regardless of an individual’s economic status or type of insurance. The Board will encourage the study of these justice issues from ethical, economic, and medical perspectives in order to advise policy makers and providers in these areas. The Ethics Committee will also consider ways of monitoring “best practices” used by other stem cell research funding agencies for balancing commercial incentives for the development of stem cell therapies with the public need for open and affordable access to new therapies.

Research integrity: Stem cell research must be carried out in full accord with standard practices for all fields of biomedical research (e.g., concerning proper conduct of research, conflicts of interest, and other areas). The Board may identify issues of unique importance in stem cell research that may benefit from further guidance.

- *Support research and scholarship on ELSIE issues as they relate to stem cell research that will help advance the research itself and inform public policy.*

The Ethics Committee may recommend that NYSTEM sponsor additional empirical or other research from fields such as philosophy, decision-making, the law, or sociology to understand current attitudes, knowledge, and practices concerning stem cell research ethics and policy. Likewise, health outcomes research and health services research efforts will be needed to address the social impact of stem cell research, including cost-effectiveness issues related to potential stem cell-based treatments. Such research may not provide answers as to what *should* be done but may better inform policy makers, researchers, health care providers, patient advocacy groups, and the public at large and help determine what areas and what audiences should be the target for educational efforts.

An example of an issue that might require research is the current debate about payment for gamete donations, including concerns that compensation could lead to poorer women disproportionately providing oocytes. No data are available to inform

this central public policy issue. Hence, an important priority would be to investigate the sociodemographic and other characteristics of women who choose to provide embryos or oocytes. If, in the future, additional compensation is provided, then data on these characteristics would be particularly important to developing policy recommendations.

- *Engage diverse communities in order to enhance public and professional understanding of critical ethical, legal, and social issues and provide opportunities for education on stem cell research and its impact on society.*

As New York State invests in stem cell research, it will be important to increase the public's awareness of the opportunities, challenges, and uncertainties of stem cell research, as well as raise awareness among stem cell scientists of the public's views and concerns. The Board will work to foster an environment in which information about stem cell research and its implications, as well as the activities of the Board, are openly communicated in a way that values accuracy and respects divergent views. Education of students (at several levels), physicians, patients, journalists, and the general public will be a priority. The particular needs of each of these audiences may vary and efforts will need to be targeted in different ways, both in form and content. Some educational initiatives may best be accomplished through funding initiatives to undergraduate institutions or other non-profit organizations. Others may be accomplished through a centralized effort conducted through the Ethics Committee in conjunction with the New York State Department of Health. Examples of learning opportunities that may be developed are: conferences targeted to scientific trainees or journalists; educational materials and slide presentations; and website-based educational content, video interviews, podcasts and stories about stem cell scientists and the latest research findings.

Initiatives targeting the specific educational needs of medical and other personnel regarding stem cell science and pathophysiology of particular diseases may be supported. In some cases, the coordinated, centralized development of educational materials may be more efficient than having multiple institutions engaged in parallel processes. For example, research on areas of misunderstanding about stem cells in the popular press could inform future efforts for education of journalists. Other programs or resources could be developed to educate scientists, attorneys, technology transfer personnel, entrepreneurs, or others on the appropriate application of intellectual property practices and commercialization of the products of stem cell research in the context of the public interest in access to the outcomes of publicly funded research.

6. Administration of the Empire State Stem Cell Trust Fund

MISSION

Administer the Empire State Stem Cell Trust Fund under the highest standards of accountability and integrity on behalf of the people of New York State.

GOALS

- Manage the NYSTEM program and administer funding in a manner that supports the mission of the Board and engenders public trust.
- Institute an independent peer review process to ensure funding of high-quality research.
- Establish contract processes to ensure the responsible conduct of research.
- Promote public access to information about stem cell research and the activities of the Empire State Stem Cell Board.

³The complete by-laws of the Empire State Stem Cell Board can be accessed at http://stemcell.ny.gov/about_esscp_bylaws.html.

Introduction

The New York State Department of Health's Wadsworth Center has been assigned responsibility, under the direction of the Board, for the administration of New York's stem cell research program, referred to as New York State Stem Cell Science/ NYSTEM. The \$600 million commitment to support stem cell research represents a substantial investment by the taxpayers and offers hope to those who suffer from debilitating diseases that could be cured or alleviated through advances in stem cell science. This commitment, trust, and hope require that the Trust Fund and its programs be administered under the highest standards of accountability and integrity on behalf of the people of the state.

Significance of the Goals for Administration of the Empire State Stem Cell Trust Fund

- *Manage the NYSTEM program and administer funding in a manner that supports the mission of the Board and engenders public trust.*

The Board's primary responsibility under the statute is to make recommendations to the Commissioner of Health regarding standards and the award of grants funded through the Trust Fund. NYSTEM staff has worked closely with the Board to ensure that the programs and policies developed by staff are in alignment with the Board's central mission. The Board has provided substantial direction to NYSTEM staff through the development of this Strategic Plan and a total of 15 meetings of the Ethics and Funding Committees in its first nine months of operation. In recognition of its need to preserve the public trust, the Board has adopted by-laws that subject its members to the highest ethical standards to avoid even the appearance of impropriety in its decisions.³ Both NYSTEM staff and the Board are committed to assuring scientific integrity, grantee accountability, and public accessibility to government decision-making. Furthermore, Wadsworth Center's ability to draw on the knowledge of its own research scientists and staff who are experienced in grant contract administration will minimize administrative expenses, while maximizing support for stem cell research and the Board's mission.

- *Institute an independent peer review process to ensure funding of high-quality research.*

The enabling statute requires the Funding Committee to provide for an independent panel of experts to review grant applications and make recommendations to the Funding Committee for the award of grants and contracts. The purpose of this requirement is to ensure that state funds are used to support only scientifically meritorious research that complies with the criteria established by the Funding Committee. The by-laws adopted by the Board establish qualifications for members of the Independent Scientific Peer Review Panels (ISPRP), standards for identifying and addressing conflicts of interest, and processes for the evaluation and compilation of recommendations to the Funding Committee.

In November 2007, the Funding Committee solicited bids for a contractor to implement the independent scientific peer review process. The selected vendor was approved by the Funding Committee at its January 7, 2008 meeting, and the contract and merit review processes were finalized in the spring of 2008. The contract specifies that all reviews will be conducted in accordance with the scoring criteria and standards approved by the Funding Committee. To ensure the scientific

merit review process is conducted by the most knowledgeable and experienced reviewers available, the selection and solicitation of ISPRP members will be conducted in concert and consultation with NYSTEM staff and the Funding Committee. NYSTEM staff will directly oversee the review process and report to the Funding Committee regarding the activities of the ISPRP and its findings.

- *Establish contract processes to ensure the responsible conduct of research.*

Wadsworth Center has substantial experience in the administration and oversight of scientific grants through the Spinal Cord Injury Research Board and Health Research Science Board. This experience, along with the Center's access to world-renowned scientists, was a significant factor in the decision to situate the NYSTEM program within Wadsworth Center. In anticipation of an increase in the number of awards, contracts, progress reports, and monitoring activities associated with this program, Wadsworth Center has recruited additional staff to ensure proper contract administration, oversight, and accountability among grantees.

The Board and staff have followed both state and federal regulatory models to ensure responsible administration of its awards and preserve the integrity of the research enterprise it supports. Grantees are required to submit regular financial accounting statements and scientific research progress reports. NYSTEM contracts require compliance with standards for the proper conduct of research, including compliance with human research subject protections, humane care and use of animals, and nationally and internationally recognized standards for hESC research, as modified by the Board. Grantees must also agree to make the results of their research available to other investigators and the public, to present their findings at NYSTEM-sponsored meetings or symposia, and to be subject to monitoring and audit of their contract expenditures.

- *Promote public access to information about stem cell research and the activities of the Empire State Stem Cell Board.*

The unique qualities of stem cell research and its potential to open new avenues for cures and therapies have sparked the public interest and scrutiny in this scientific endeavor in ways that have rarely occurred with other scientific initiatives. In response to this level of interest, the Board is committed to supporting programs and policies that enhance the public's understanding of stem cell research, its opportunities, challenges, and uncertainties. Additionally, it is committed to ensuring public access to information regarding the Board's activities and the programs and research supported with public funds.

The meetings of the Board and its Committees are subject to the Open Meetings Law, and the Board is required to issue an annual report to the public that includes information about its activities, grants awarded, grants in progress, research accomplishments, administrative expenses, future program directions, and the Strategic Plan. The Board and staff have exceeded these mandates in promoting public access to the Board and its activities by establishing a website - www.stemcell.ny.gov - that includes meeting notices, agendas, minutes, reports, publications, Board presentations, grant opportunities, awards, research findings, information about Board members, and other relevant information. The website also provides links to live and recent webcasts of all Board and Committee meetings and allows stakeholders to sign up for e-alerts regarding upcoming Board events, announcements, and funding opportunities. Under the guidance of the Board, the website will be enriched with additional information about stem cell research, such as lay summaries of the latest research findings, video interviews with New York State scientists, and links to other information and educational materials. Other opportunities for increasing the exchange of information, ideas, and views about stem cell science among the public, scientists, and stakeholders will also be pursued.

7. Benefits of promoting stem cell research in New York, evaluation, and public accountability

MISSION

Ensure New York State preeminence in the application of knowledge derived from stem cell research for the greater public good and the generation of long-term support for stem cell research.

GOALS

- Annually assess progress toward achieving the goals of the Strategic Plan and identify potential modifications as part of the Board's annual report to the public.
- Evaluate intellectual property, technology transfer, and fiscal policies to ensure broad access to the results of state-funded research and optimum benefits to New York State and its residents.
- Assess the economic and other benefits derived from the state's investment in stem cell research at regular intervals, maximize support for stem cell research by funding high-quality science, and use state funds to leverage additional commitments to stem cell research in New York.

Introduction

New York State has committed \$600 million to stem cell research because of the potential it holds for helping millions of New Yorkers suffering with debilitating diseases and conditions. This commitment represents New York's stake in an exciting research frontier that has the potential to transform health care, as pharmaceuticals and organ and tissue transplants have done previously. Undoubtedly, New Yorkers hope this investment will deliver concrete results that propel the science forward and yield clinical applications in the future. However, the uncertainty of scientific exploration makes it impossible to predict the precise role New York will play in developing new stem cell-based therapies or cures, or the direct benefit to individual New Yorkers suffering from disease. Nonetheless, this investment ensures that New York will play a prominent role in advancing the understanding of stem cell science in ways that will help the scientific community discern which avenues of inquiry are likely to yield the most beneficial results. Thus, New York's commitment is expected to have a sizeable and sustained impact on the future of stem cell science and the potential development of therapies and cures that will benefit the residents of New York State and beyond.

Although not a primary goal of this initiative, this investment is expected to yield economic benefits for the State. By funding high-quality stem cell research, New York will attract some of the best and brightest minds in biomedical research and lay the foundation for a stronger, more dynamic biotechnology research and business community within the State. The infusion of funds, talent, and new energy into the State's scientific research community is expected to encourage investments far beyond those appropriated by the State. As stewards of the public monies and the promise of this scientific revolution, the Board and NYSTEM staff will continually assess the efficacy and efficiency of the State's policies, programs, and investments to ensure public accountability.

Significance of the Goals for Promoting Stem Cell Research in New York, Evaluation, and Public Accountability

- *Annually assess progress toward achieving the goals of the Strategic Plan and identify potential modifications as part of the Board's annual report to the public.*

This Strategic Plan is the foundation for the Board's decisions on the allocation of funds and future program directions. As part of its annual report, the Board will assess its progress in meeting the goals established in this Plan and inform the public of the accomplishments of state-funded research. Since stem cell science is a rapidly evolving field, it is impossible to predict which research avenues and investments will be most fruitful. For this reason, the Board will reassess this Strategic Plan annually to examine current understanding of stem cell science and to redirect priorities into areas that will accelerate knowledge and encourage the development of new approaches to treat and cure disease. The status of infrastructure, training and education programs, ethical policies and practices, and other tools to support and guide New York's stem cell research community in reaching its full potential will be evaluated. Thus, this Strategic Plan is a "living document" that will routinely be reviewed and revised in the light of developing

knowledge under the critical eye of the Board. Every effort will be made to ensure that the State's funds support the best and most promising science in ways that will maximize the benefits to the public and encourage ongoing support for stem cell research in New York State. Because the enabling statute requires that the Board issue an annual report on its activities, grants, research accomplishments, and future program directions, the annual evaluation of the Strategic Plan will be a natural core component of the Board's report.

- *Evaluate intellectual property, technology transfer, and fiscal policies to ensure broad access to the results of state-funded research and optimum benefits to New York State and its residents.*

The statute creating the Board requires that all grants be subject to intellectual property agreements that set forth the scope, if any, of the State's financial or other interests in the commercialization of the results, products, inventions, and discoveries emanating from state-funded research. In keeping with this mandate, the Funding Committee has imposed several requirements applicable to intellectual property rights. Principal among them are standards assuring public access to the published results of state-funded research, sharing of research-developed products and methodologies among the research community, and the right of the State to protect or commercialize state-funded discoveries that may be patentable and marketable if the grantee fails to do so. The Board also has considered California's complex regulatory framework that requires a return on the State's investment if certain conditions are met. This approach requires extensive recordkeeping and other obligations viewed as being unduly burdensome, given that a significant return of the State's investment is unlikely in most cases. Such policies may discourage, rather than encourage, translational research and needed collaborations with industry. Other mechanisms might permit the State to participate in potential windfall profits derived from state-funded research without creating disincentives. Consequently, the Board will continue to explore these issues and attempt to identify creative ways to derive benefits for the residents of the State without undermining the mission and goals of the Board and the underlying stem cell research program.

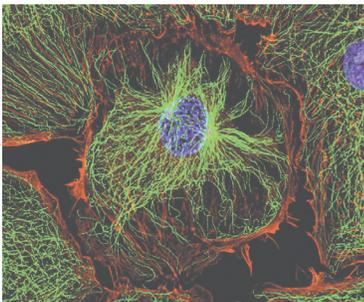
- *Assess the economic and other benefits derived from the State's investment in stem cell research at regular intervals, maximize support for stem cell research by funding high-quality science, and use state funds to leverage additional commitments to stem cell research in New York.*

The legislation creating the Board and the Trust Fund intentionally did not make economic development the centerpiece, or even an explicit goal, of the enabling legislation. Nonetheless, this targeted investment in stem cell research will undoubtedly provide economic benefits to the State and its residents. The infusion of funds directed at high-quality, cutting-edge research will attract and support great science and great scientists. This, in turn, will attract outside research funding and create a fertile ground for expansion of New York's biotechnology industry. The Board's mission statement reflects the fact that the NYSTEM program can only fulfill its goal of accelerating the growth of stem cell science and the development of therapies and diagnostic methods by fostering a robust stem cell research community. Promoting a strong research environment will require infrastructure, talent, training opportunities, and resources that by their very nature create new jobs, business opportunities, and sources of revenues, which are hallmarks of economic development. At the same time, the development of a strong stem cell research community requires and is expected to stimulate funding beyond that provided

by the State. The program is likely to increase investment in New York stem cell research from academia, philanthropic organizations, the business community, and the residents of the State. Thus, it is a foregone conclusion that the NYSTEM program will enjoy a symbiotic relationship with economic development in New York State.

Regular evaluation of all benefits and outcomes of the NYSTEM program, including any potential economic impact, will enable the Board to make informed choices about program priorities and directions and to fulfill its responsibility for public accountability. Consequently, the Board, with the assistance of outside experts, will undertake a study to assess the economic benefits of this initiative in the second, sixth, and tenth year of the program. The initial and ongoing assessment will monitor activities and funding in a wide variety of areas pertaining to stem cell research including, but not limited to:

- Extramural grants and contracts awarded for stem cell research
- Funding for stem research at individual research entities in New York State
- Combined funding for stem cell research at New York State research entities
- New funding from any public or private source obtained as a result of NYSTEM-funded research
- Number of stem cell scientists recruited to New York State institutions
- Overall number of faculty conducting stem cell research in New York State
- Number of undergraduate, graduate, post-graduate, and medical students pursuing stem cell-related training or careers
- Stem cell laboratory employment
- Funding from out-of-state collaborations
- Patents and licenses issued to funded stem cell scientists
- Number of stem-cell related biotechnology companies in New York State and their revenues
- Major recognition/prizes awarded to New York State stem cell researchers
- Landmark stem cell publications from New York researchers
- New buildings, laboratories, and shared facilities that support stem cell research
- New clinically relevant biological and biochemical targets for stem cell therapy
- Clinical stem cell trials initiated
- Clinical stem cell trials completed
- New clinically effective stem cell treatment regimens developed



Every effort will be made to ensure that the NYSTEM program proceeds according to plan and is successful in the areas delineated above. It is anticipated that education of elected officials and the public about progress and the promise of stem cell research in New York State will result in continued public support for stem cell research. With a successful NYSTEM program and strong public support for stem cell research, New York can emerge as one of the preeminent regions in the world for stem cell research and biotechnology development.

Appendix I: Roster of the Empire State Stem Cell Board

Richard F. Daines, M.D., Chair*‡

Commissioner
New York State Department of Health

David C. Hohn, M.D., Vice Chair*‡

President Emeritus and Executive Director of Health Policy
Roswell Park Cancer Institute

Kenneth Adams, M.B.A.*

President and CEO
The Business Council of New York State

Fr. Thomas Vincent Berg, LC, Ph.D. ‡

Executive Director
The Westchester Institute

Bradford C. Berk, M.D., Ph.D.*

Senior Vice President for Health Sciences
CEO of the Medical Center and Strong Health
University of Rochester Medical Center

Nancy Neveloff Dubler, LL.B. ‡

Director, Division of Bioethics
Montefiore Medical Center
Professor of Bioethics
Albert Einstein College of Medicine

Richard Dutton, Ph.D.*

Member
Trudeau Institute

Robin Elliott, M.A.*

Executive Director
Parkinson's Disease Foundation

Brooke Mackenzie Ellison, M.A. ‡

Founder and President
Brooke Ellison Project

Gerald Fischbach, M.D.*

Scientific Director, Simons Foundation Autism Research Initiative
Executive Vice President Emeritus
Health and Biomedical Sciences
Columbia University

Daniel Sulmasy, O.F.M., M.D., Ph.D. ‡

Professor of Medicine and Director of the Bioethics Institute
New York Medical College
Sisters of Charity Chair in Ethics
Saint Vincent's Hospital Manhattan

Robert N. Swidler, M.A., J.D. ‡

General Counsel
Northeast Health

Samuel Gorovitz, Ph.D. ‡

Founding Director
Renée Crown University Honors Program
Professor of Philosophy
Syracuse University

Bruce A. Holm, Ph.D.*

Senior Vice Provost
State University of New York at Buffalo
Executive Director
NYS Center of Excellence in
Bioinformatics & Life Sciences

Hilda Y. Hutcherson, M.D., FACOG*

Clinical Professor
Department of Obstetrics and Gynecology
Columbia University

Robert Klitzman, M.D. ‡

Associate Professor of Clinical Psychiatry
Department of Psychiatry
Columbia University College of Physicians and Surgeons

Vivian S. Lee, M.D., Ph.D, M.B.A‡

Professor and Vice Chair for Research
Department of Radiology
Vice Dean for Science, Senior Vice President
and Chief Scientific Officer
New York University Medical Center

Rev. H. Hugh Maynard-Reid, D. Min., BCC, CASAC‡

Director of Pastoral Care Services
North Brooklyn Health Network
Woodhull Medical Center

Rev. Msgr. William B. Smith, S.T.D.‡

Faculty Member
St. Joseph's Seminary

Michael Stocker, M.D., M.P.H.*

Member, Board of Overseers
Albert Einstein College of Medicine
Retired CEO, Empire Blue Cross/Blue Shield

Harold Varmus, M.D.*

President
Memorial Sloan-Kettering Cancer Center

Madelyn Wils *

Executive Vice President
New York City Economic Development Corporation

* member of the Funding Committee

‡ member of the Ethics Committee

Appendix 2: Strategic planning process

The Empire State Stem Cell Board Strategic Plan was developed through an iterative, collaborative process that involved members from both Committees of the Board, as well as external stem cell research experts and NYSTEM staff. At its first meeting, the Board was presented with a staff report summarizing the nature and extent of New York State stem cell research as of mid-2007,⁴ and input from the scientific community, based on surveys of and face-to-face interviews with State stem cell scientists. Subsequently, a Strategic Plan Coordinating Committee (SPCC) was charged with developing a mission statement, program goals, and a plan for evaluation and public accountability as the basis for an initial draft of the Strategic Plan. The SPCC held seven in-person and teleconference meetings from December 2007 to March 2008 to discuss the structure and content of the Strategic Plan. Dr. Fischbach and Dr. Klitzman developed initial draft outlines that reflected the viewpoints of the Funding Committee and Ethics Committee, respectively. Following that, individual members were tasked with drafting specific chapters of the Plan, with the assistance of Dr. Michelle Cissell, a scientific writer who had been retained at the direction of the SPCC. In addition, an Ethics Workgroup was formed to refine the elements of the Strategic Plan related to ethical, legal, and social issues and education. The Funding and Ethics Committees of the Board separately discussed the strategic planning process and commented on preliminary drafts of the Strategic Plan at 11 meetings held from October 2007 through May 2008. Comments provided at the Board meetings were used by the SPCC members to further develop and hone the Strategic Plan.

STRATEGIC PLAN COORDINATING COMMITTEE

Michael A. Stocker, M.D., M.P.H., Chair

Member, Board of Overseers
Albert Einstein College of Medicine
Retired CEO, Empire Blue Cross/Blue Shield

Harold Varmus, M.D., Vice Chair

President
Memorial Sloan-Kettering Cancer Center

Gerald Fischbach, M.D.

Scientific Director, Simons Foundation Autism Research Initiative
Executive Vice President Emeritus
Health and Biomedical Sciences
Columbia University

David C. Hohn, M.D.

President Emeritus and Executive Director of Health Policy
Roswell Park Cancer Institute

Robert Klitzman, M.D.

Associate Professor of Clinical Psychiatry
Department of Psychiatry
Columbia University College of Physicians and Surgeons

Susan L. Solomon

Chief Executive Officer
New York Stem Cell Foundation

Allen M. Spiegel, M.D.

Marilyn and Stanley M. Katz Dean
Albert Einstein College of Medicine

STRATEGIC PLAN ETHICS WORKGROUP

Fr. Thomas Vincent Berg, LC, Ph.D.

Executive Director
The Westchester Institute

Nancy Neveloff Dubler, LL.B.

Director, Division of Bioethics
Montefiore Medical Center
Professor of Bioethics
Albert Einstein College of Medicine

Brooke Mackenzie Ellison, M.A.

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Brooke Ellison Project

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Associate Professor of Clinical Psychiatry
Department of Psychiatry
Columbia University College of Physicians and Surgeons

Michael Stocker, M.D., M.P.H.

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Albert Einstein College of Medicine
Retired CEO, Empire Blue Cross/Blue Shield

⁴Stem Cell Research in New York State: A Snapshot can be accessed at: http://stemcell.ny.gov/publications_stem_cell_research_in_nys.html

Appendix 3: Glossary of terms and abbreviations

Adult (or somatic) stem cell	An undifferentiated cell found in a differentiated tissue that can renew itself and differentiate (with certain limitations) to give rise to all the specialized cell types of the tissue from which it originated. It is important to note that scientists do not agree about whether or not adult stem cells may give rise to cell types other than those of the tissue from which they originate.
Blastocyst	A preimplantation embryo of about 150 cells produced by cell division following fertilization. The blastocyst is a sphere made up of an outer layer of cells (the trophoblast), a fluid-filled cavity (the blastocoel), and a cluster of cells on the interior (the inner cell mass).
Cell-based therapies	Treatment in which stem cells are induced to differentiate into the specific cell type required to repair damaged or destroyed cells or tissues.
Chimera	An organism composed of cells derived from at least two genetically different cell types. The cells could be from the same or separate species.±
Clone	Generate identical copies of a molecule, cell, or organism. 1. When it is used to refer to cells grown in a tissue culture dish, a clone is a line of cells that is genetically identical to the originating cell. This cloned line is produced by cell division of the originating cell. 2. The term clone may also be used to refer to an animal produced by somatic cell nuclear transfer (SCNT).
Differentiation	The process whereby an undifferentiated embryonic cell acquires the features of a specialized cell such as a heart, liver, or muscle cell.
Directed differentiation	Manipulating stem cell culture conditions to induce differentiation into a particular cell type.
Embryo	In humans, the developing organism from the time of fertilization until the end of the eighth week of gestation, when it is called a fetus.
Embryonic stem cells	Primitive (undifferentiated) cells derived from a 5-day preimplantation embryo that have the potential to become a wide variety of specialized cell types.
Embryonic stem cell line	Embryonic stem cells, which have been cultured under <i>in vitro</i> conditions that allow proliferation without differentiation for months to years.
Enucleated	A cell with its nucleus removed.
Fertilization	The joining of the male gamete (sperm) and the female gamete (egg).
Gamete	An egg (in the female) or sperm (in the male) cell.
Induced pluripotent stem cell	A form of pluripotent stem cell that is created in the laboratory by reprogramming differentiated cells into an embryo-like state by using viruses to insert new genes.³
Inner cell mass	The cluster of cells inside the blastocyst. These cells give rise to the embryo and ultimately the fetus. The inner cell mass cells are used to generate embryonic stem cells.
Mesenchymal stem cell	Also known as bone marrow stromal cells, mesenchymal stem cells are rare cells, mainly found in the bone marrow, that can give rise to a large number of tissue types such as bone, cartilage (the lining of joints), fat tissue, and connective tissue (tissue that is in between organs and structures in the body).°
Morula	A solid mass of 16–32 cells that resembles a mulberry and results from the cleavage (cell division without growth) of a zygote (fertilized egg).±
Multipotent	Ability of a single stem cell to develop into more than one cell type of the body. (See also pluripotent and totipotent.)

Preimplantation	With regard to an embryo, preimplantation means that the embryo has not yet implanted in the wall of the uterus. Human embryonic stem cells are derived from preimplantation stage embryos fertilized outside a woman's body (<i>in vitro</i>).
Regenerative medicine	A treatment in which stem cells are induced to differentiate into the specific cell type required to repair damaged or destroyed cell populations or tissues. (See also cell-based therapies.)
Reproductive cloning	The goal of reproductive cloning is to create an animal being identical to the animal that donated the somatic cell nucleus. The embryo is implanted in a uterus and develops into a live being. The first animal to be created by reproductive cloning was Dolly the sheep, born at the Roslin Institute in Scotland in 1996. [NOTE: The Empire State Stem Cell Board statute specifically prohibits support for research that directly or indirectly involves human reproductive cloning.]
Somatic cell	Any body cell other than gametes (egg or sperm).
Somatic cell nuclear transfer (SCNT)	A technique that combines an enucleated egg (nucleus removed) and the nucleus of a somatic cell to make an embryo. SCNT allows the reprogramming of adult cells into disease-specific embryonic stem cell lines that have potential for the study and treatment of diseases.
Stem Cells	Cells with the ability to divide for indefinite periods in culture and to give rise to specialized cells.
Totipotent	A totipotent stem cell can give rise to all the cell types that make up the body plus all of the cell types that make up the extraembryonic tissues such as the placenta. (See also pluripotent and multipotent.)
Umbilical cord blood stem cells	Stem cells collected from the umbilical cord at birth that can produce all of the blood cells in the body (hematopoietic). Cord blood is currently used to treat patients who have undergone chemotherapy to destroy their bone marrow due to cancer or other blood-related disorders.
Undifferentiated	A cell that has not yet generated structures or manufactured proteins characteristic of a specialized cell type.

Source: National Institutes of Health, except as noted

^o Source: International Society for Stem Cell Research

[±] Source: National Academies of Science Guidelines for Human Embryonic Stem Cell Research, 2005 edition

▫ Adapted from: Baker, M. (2007) *Nature Rep Stem Cells*. <http://www.nature.com/stemcells/2007/07/200712/071206/full/stemcells.2007.124.html>

ABBREVIATIONS

ALS	amyotrophic lateral sclerosis
ANT	altered nuclear transfer
ELSIE	ethical, legal, and social issues and education
ESCRO	Embryonic Stem Cell Research Oversight Committee
FY	fiscal year
GMP	good manufacturing practice
hESC	human embryonic stem cells
iPS	induced pluripotent stem cells
ISPRP	Independent Scientific Peer Review Panel
ISSCR	International Society for Stem Cell Research
IVF	<i>in vitro</i> fertilization
NAS	National Academy of Sciences
NIH	National Institutes of Health
NYSTEM	New York State Stem Cell Science Program
RFA	Request for Applications
SCNT	somatic cell nuclear transfer

Appendix 4: Selected resources for more information on stem cell research

- **The New York State Stem Cell Science** website presents information on the activities of the Empire State Stem Cell Board, NYSTEM funding opportunities, and research supported by the program.

<http://stemcell.ny.gov/index.html>

- **The National Institutes of Health Resource for Stem Cell Research** website provides basic information on stem cell biology, an overview of stem cell research progress, information on ethical issues in stem cell research, and an explanation of the U.S. policy on stem cell research.

<http://stemcells.nih.gov/index.asp>

- **The Stem Cells at the National Academies** website offers reports and publications on guidelines for human embryonic stem cell research, stem cell basics, regenerative medicine, and related issues.

<http://dels.nas.edu/bls/stemcells/>

Acknowledgements

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Science Writer and Consultant

Michelle A. Cissell, Ph.D.

M.A. Cissell Consulting

NYSTEM Staff

Lawrence S. Sturman, M.D., Ph.D.

Executive Director

Judy L. Doeschate, J.D.

Director of Board Operations

David G. Anders, Ph.D.

Scientific Officer

Marti McHugh

Program Development Officer

Katherine S. Zdeb

Communications Manager



State of New York
Department of Health