# INSTITUTE

#### A PUBLICATION OF THE NEURAL STEM CELL INSTITUTE

Second Edition, Spring 2015



#### Stem cell research to cure nervous system disease...

The Neural Stem Cell Institute (NSCI) is dedicated to developing regenerative stem cell therapies for diseases of the central nervous system including Parkinson's, Alzheimer's, Macular Degeneration, Autism and Spinal Cord Injury. Led by MacArthur "Genius" Award winner Dr. Sally Temple, who helped discover and define nervous system stem cells, NSCI is the first independent, nonprofit stem cell research institute in the United States.

#### Disease statistics that drive our commitment...

- Approximately 60,000 Americans are diagnosed with Parkinson's each year
- 1 in 68 children are diagnosed with Autism
- Women in their 60s are twice as likely to develop Alzheimer's than breast cancer
- Over 10 million people suffer from Macular Degeneration
- Motor Vehicle accidents account for 46% of spinal cord injuries
- **Multiple Sclerosis** is the most widespread disabling neurological condition in young adults around the world

#### **NEW & NOTEWORTHY**

## Dr. Sally Temple Awarded Defense Department Grant to Study Neurofibromatosis

In late February Dr. Sally Temple learned she had won a threeyear \$713,384 grant to study neurofibromatosis 2. The Department of Defense office of Congressionally Directed Medical Research Programs awarded the grant based on Dr. Temple's application, *Modeling NF2 Tumors for Drug Screening Using Induced Pluripotent Stem Cells*.

The grant will fund the study of neurofibromatosis 2, a disease that causes tumors to grow in the nervous system commonly resulting in pain and loss of hearing. The disease can be life threatening.

"We are thrilled to be able to work on this [disease state]," said Dr. Temple. "We are collaborating with Dr. Deanna Thompson at Rensselaer Polytechnic Institute."

NSCI Board Member Vito Grasso explained the significance of the award. "This is an extraordinary development for NSCI and for stem cell research. The DOD Congressionally directed medical research program for NF2 is closely monitored by NF patients, their families and researchers," said Grasso. "This project will help integrate NSCI into the NF community thereby establishing a new and important foundation for NSCI and stem cell research to become linked to health and diseasespecific organizations. This relationship can help immeasurably in promoting support for stem cell research and dissemination of important research findings."

The Neurofibromatosis Research Program (NFRP) was initiated by the Defense Department in 1996 to provide support for research of exceptional scientific merit that promotes the understanding, diagnosis and treatment of neurofibromatosis. The vision of the NFRP is to decrease the clinical impact of NF. "Toward this end, the NFRP seeks to support innovative, high-impact



Dr. Sally Temple

research that will foster new directions for and address neglected issues in NF research; sponsor multidisciplinary and multiinstitutional collaborations that will bring new perspectives to the field; promote translational and clinical studies to move promising ideas from bench to bedside; and develop a balanced portfolio of meritorious research related to all aspects of neurofibromatosis."

## Neural Stem Cell Institute Co-Founder Dr. Sally Temple Elected Vice President of International Society for Stem Cell Research



Dr. Sally Temple was elected Vice President of the International Society for Stem Cell Research

(ISSCR) in mid-2014. ISSCR is an independent nonprofit organization and the voice of global stem cell research. Founded in 2002 to foster the exchange of information and opportunities for new discoveries in stem cell science, ISSCR has over 4,100 members worldwide.

Dr. Temple will have a global impact on the stem cell community as Vice President of ISSCR. She hopes to be instrumental in forwarding the momentum of research. "This is a truly exciting era for stem cell science and regenerative medicine, and ISSCR plays a key role in bringing together multidisciplinary research and clinical constituencies from around the world," says Dr. Temple. "One of the most inspiring achievements of stem cell research is turning science into new patient therapies. I will help ensure that ISSCR provides a clear message and vital support for the quality basic and translational research needed to secure the promise of new therapies for sick patients."

Dr. Temple founded NSCI with the dream of using stem cells to cure diseases of the nervous system. Her position at ISSCR is more validation of how important her work has been and continues to be. "Dr. Temple is a rock star in the stem cell community. Her peers and colleagues respect her immensely. As Vice President of ISSCR, I am confident that she will continue to steer us on the right path," says Dr. Christopher Fasano, Director of Research and Development at NSCI.



## "The Stem Cell Podcast" Named Official Podcast of the ISSCR

NSCI is proud to announce that *The Stem Cell Podcast* has become the official podcast of The International Society for Stem Cell Research (ISSCR). Launched in September 2013, *The Stem Cell Podcast* was developed and is co-hosted by Dr. Chris Fasano, a principal investigator and Director of Research and Development at NSCI. Go to www.stemcellpodcast.com to hear all episodes.

ISSCR is an independent nonprofit organization and the voice of the stem cell research community. The ISSCR was founded in 2002 to foster the exchange of information on stem cell research and now claims more than 4,100 members worldwide.



## Dr. Chris Fasano Guest Speaker at The College of St. Rose

The College of St. Rose in Albany, NY, has invited Dr. Chris Fasano to address its developmental biology and genetics classes. NSCI is excited about this new relationship and pleased to contribute to the college's growing science department. Dr. Fasano is Director of Research and Development at NSCI.



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## Year Three of the Retinal Stem Cell Consortium... Closer to a Cure

by Michael Naimark, Project Manager

The Retinal Stem Cell Consortium (RSCC) was created with the goal of developing a stem cell-based clinical therapy for the treatment of Age-Related Macular Degeneration (AMD), the leading cause of blindness in people over age 55.

AMD affects approximately 10 million patients in the U.S. Current treatments are limited, and the FDA does not recognize any effective therapy. The RSCC is focused on developing a truly viable treatment based on novel technology pioneered by the Neural Stem Cell Institute under the guidance of Director Dr. Sally Temple and co-founder Dr. Jeffrey Stern.

Now in its third year of operation, the consortium has made great progress toward that goal.

A project of this nature presents a series of challenges that must be confronted as each phase unfolds. Since the goal is to develop a patient treatment for AMD and bring it into clinical trials by the end of the fourth year, each obstacle must be addressed quickly and efficiently.

In the first year of the project many of the major hurdles were logistical, such as coordinating the efforts of the six participating research institutions. By breaking down the critical components of the project among the teams at the NSCI, The University at Albany, Fordham University, Burke Medical Research Institute, Mt. Sinai Medical Center and the University of Rochester, the RSCC was able to establish a framework for successful progress.

Many exciting developments occurred in the second year of the project, including our first direct meeting with FDA representatives from the Center for Biologics Evaluation and Research (CBER) and the Office of Cellular, Tissue and Gene Therapies (OCTGT). We were able to present our preliminary data and research objectives to the administrators and scientists who will ultimately be responsible for the final review of any RSCC-developed therapy.

Based on the FDA's feedback, we moved forward with confidence during the second year of the project. For the first time, we were able to demonstrate that our stem cell technology could effectively and consistently preserve vision in living animals. We made several unexpected discoveries about how our stem cells behaved at various stages of growth, and how different growing conditions influenced their potency as a treatment. These discoveries not only helped our team create a more effective therapy for clinical use, they also expanded what we know about stem cell growth and behavior, and will help other scientists around the world in developing their own therapies.

We also began our first preliminary safety studies during the second year. These mandatory safety studies must be performed under a very stringent set of regulations. They are the most important piece of information the FDA considers when reviewing a new therapy. Our first pilot safety study was successfully completed in early 2015.

We have several critical milestones to meet in our third year, as we continue to target early 2017 for the first human clinical trials of our AMD therapy. This will be the year when we apply what we've discovered to designing and running critical studies that will yield key data required by the FDA. We will meet with FDA representatives again soon to review our most recent results and lay out proposals for the most important studies of the project. These will include our largest, longest study to confirm the continued safety of our cell therapy under FDA regulations.

After extensive refinement of cell manufacturing processes at their facility, our partners at the University of Rochester will be establishing their first FDA-compliant master banks of our cells for use in eventual clinical trials. Each cell bank is projected to contain enough material to treat more than 300 patients.

Meanwhile, our expert visual testing partners at Burke Medical Research Institute will be performing their most in-depth investigation of sight preservation in animals to date using multiple methods of analysis. The goal is to demonstrate to the FDA that our cell treatment provides significant and long-lasting benefits.

While the RSCC has already made great advances in stem cell technology, the studies we perform this year will be crucial. They hold the key to proving that we can produce an effective treatment for a degenerative disease that impacts tens of millions of people worldwide.

This year promises to be the most exciting one yet, as we prepare for a final push toward FDA approval of our AMD treatment. In that effort, we thank our combined roster of 33 dedicated scientists and technicians, the community and our supporters. The RSCC looks forward to making "Year 3" of the project a successful and rewarding one.



Dr. Jeffrey Stern

## NABA Names NSCI Co-Founder Dr. Jeffrey Stern "Professional of the Year"

Interview by Suzanne Kawola

The Northeastern Association of the Blind at Albany (NABA) is recognizing NSCI's Dr. Jeffrey Stern as *Professional of the Year* at its annual Visionary Gala on April 18, 2015, at Franklin Plaza in Troy, NY.

"Jeff has been a longstanding member of the NABA family," says Christopher Burke, Executive Director of NABA. "He's always been a supporter of NABA. Being a rehab agency, we rely on doctors for referrals for people to come here for care. And, Jeff and his office have always been very forthcoming with referring people to us for services, which is not always an easy thing, because by the time a doctor is referring someone to NABA basically they are saying there isn't anything medically they can do. That is a very difficult message to deliver, but it's incredibly necessary. Jeff has always been willing to make those referrals, which means he really cares about his patients. He understands what NABA does and understands the partnership in that continuum and he participates in it and that is really a wonderful thing. He's a role model for other doctors."

Burke explains his enthusiasm for NSCI's research: "We also are very supportive of the research that is being done; his efforts and Sally's [Temple] efforts to move forward with stem cell research. It's very important to the people that we serve if there are breakthroughs or forward movement. It's going to benefit our people tremendously. And, it's locally based so we are very supportive and interested in what they are doing."



Burke illustrates the collaborative nature of NABA's mission: "Not everyone who influences us has to be a board member or committee member. We rely on other organizations. We rely on people in the community to say, 'this is where I think you should be putting your services, or here's someone I think you should be reaching out to, or here's an area that is underserved.' Those pieces of information come from people like Jeff who are active. He has always had

Christopher Burke

something to tell me, some insight, some piece that helps NABA move along. He's paying attention to NABA and paying attention to what his patients need."

NABA was established in Albany in 1908. It currently serves 14 counties. The largest population served by NABA is senior citizens, however, all ages are represented including infants and children. NABA regularly recognizes its many partners in the community, but its *Professional of the Year Award* is its most prestigious. NABA partners with organizations where there could potentially be a need for vision impairment support services.

"We have to let people know that we are here and our services exist," explains Burke. One of NABA's biggest challenges is making people aware of its array of services for the blind and visually impaired. "There are many people out there who are losing their vision and won't recognize it or try to work around it and not deal with it directly. We have to get to those people and get them into services."

Seniors with macular degeneration, glaucoma and diabetic retinopathy are among the largest populations served by NABA. "How is someone who is dealing with vision loss going to stay in their home and remain independent? That is something different for everybody," says Burke. "The adjustment can be very difficult for people, to be perfectly healthy your whole life, now you can't get around in your own house because you can't see. That's where NABA comes in."

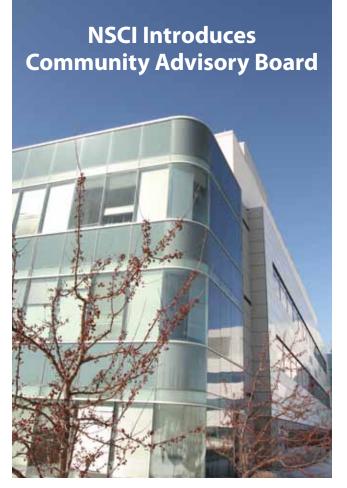
NABA is also working more with children now. As advances in technology for premature births expand, the need for services for these children is there.

NABA is a New York State certified contractor. It is certified for a myriad of services, from low vision exams, to employment services, to daily living skills. The state is a great partner, notes Burke. It provides other services that someone might need going through this life change, like emotional counseling.

The only challenge with the state is the partial funding NABA receives for vision services, according to Burke. NABA makes up the difference through fundraising and other sources of revenue. It is one of 17 certified agencies in New York for vision impairment services. And, New York is number one in providing services for the visually impaired nationwide, with the strongest legislation for these services.

NABA has expanded in the past 10 years. Says Burke: "What are the needs? How can we meet them? How do we fund it? How do we grow? As we are growing those services and reaching more people, we're also expanding our reach for support."

For tickets to NABA's Visionary Gala on April 18th, contact Michele Puleo O'Hare at (518) 463-1211 x241 or Gail Hessney at x201.



This past February 25 was the first meeting of NSCI's newly formed Community Advisory Board. The following members were in attendance: Lisa Bueneau, Jeffrey DiNapoli, Michael DiScipio, Chris Fasano, Sharon Fenno, Dan Fitzpatrick, Charlie Joseph, Suzanne Kawola, Jessica Le, Lisa Payne and Sally Temple. The meeting was held at NSCI headquarters and sponsored by Community Board member Jeff DiNapoli.

The mission of the board is to bring members' individual business experiences to bear on the growth of NSCI. Since the doors opened in 2007, the institute has made steady progress by hiring and training top-level stem cell scientists and producing world-renowned discoveries. It is a testament to the vision of the leadership, the dedication of the scientists and the independence of the organization.

"NSCI is on the cutting edge of neural stem cell research, and it is time to share news with the Capital Region and beyond," says Dr. Chris Fasano. "We must ensure that NSCI continues to grow. And, we cannot do it alone. It's time to spread the word and invite more people to see and hear about our work firsthand. We welcome to the NSCI team volunteers to help engage the community. We will implement a community outreach strategy. Together, we'll continue the quest to find new cures for devastating nervous system disorders."

Input from the first meeting held in February is setting the stage for increased visibility, resources and support for NSCI development.

## Did you know?

- NSCI scientists were the first to discover that the nervous system contains neural stem cells.
- NSCI scientists discovered a method to activate the neural stem cells that are present but normally "asleep" in the adult nervous system. Once activated, these cells mediate repair and regeneration of damaged central nervous system tissues, including spinal cord, optic nerve, retina and brain due to injury, trauma, stroke, Multiple Sclerosis, Parkinson's and Alzheimer's.
- NSCI scientists discovered a new neural stem cell present in the adult retina that self-renews to produce large numbers of progeny-enabling replacement therapies, disease modeling and drug discovery using a patient's own cells.
- NSCI scientists discovered a new model of early brain development to study neurodevelopmental diseases such as Autism.
- NSCI has received 11 National Institute of Health Awards.
- NSCI received a \$10.6 million grant from NYSTEM in 2012.

#### In just 7 years...

- · Over 33 publications in top tier journals
- Multiple patents
- Successful launch of a biotechnology company, StemCulture LLC, to sell stem cell reagents
- Creation of a retinal therapeutic company, Athghin Biotech, for eye therapies
- Creation of a stem cell conference geared toward up-andcoming investigators, the NextGen Stem Cell Conference

NSCI's Dr. Tom Kiehl will be speaking on current topics in stem cell research for brain injury at the Annual Conference of the Brain Injury Association of New York State (BIANYS) on June 5, 2015, at the Marriott Hotel in Albany, NY.



## Dr. Sally Temple's Lecture and Travel Schedule



#### 2015

**Cold Spring Harbor Laboratory,** Meeting on Stem Cell Biology, *Creating Patient-Specific Neural Cells for the In Vitro Study of Brain Disorders,* Cold Spring Harbor, NY (April)

NYSTEM Annual Meeting, New York, NY (May)

ISSCR 13th Annual Meeting, Sweden (June)

**Cornell University,** Bi-annual Cornell Stem Cell Symposium, Ithaca, NY (June)

#### 2014

**Broad Foundation** Seminar in Neurobiology, Duke University, North Carolina (January)

**Rainwater Charitable Foundation,** Futures Program with the Tau Consortium, California (January)

ISSCR 12th Annual Meeting, Vancouver, Canada (June)

**XVIth International Symposium on Retinal Degeneration,** Keynote Speaker, Pacific Grove, CA (July)

Michigan Epigenetic, Stem Cell, and Regenerative Medicine Symposium (September)

**US Society for Experimental Biology and Medicine,** Speaker at the 2014 International Experimental Biology and Medicine Conference on "Stem Cell Biology and Human Diseases," Shanghai (October)

## How Can I Help?

- Stay informed. Stem cell research is one of the fastest growing fields of scientific study. Visit www.neuralsci.org to read and receive updates on our progress.
- Visit our labs. By seeing our research up close and hearing from scientists about the potential of stem cells for medical applications, we can widen the conversation and increase support for this emerging area of neuroscience.
- Become a contributor. Future discoveries in neural stem cell research depend on the support of the community. Donations enable us to expedite new therapies from the laboratory to the patient.
- NSCI is a 501(c)(3) not-for-profit, which accepts charitable donations. We are grateful for all donations and pride ourselves on putting over 95% of those funds toward scientific research (versus administrative or fundraising). This truly makes NSCI unique.
- For more information, please visit our website, email suzannekawola@neuralsci.org or call (518) 694-8188.

Find Us Online www.neuralsci.org Twitter: @neuralstemcells Facebook: NeuralStemCellInstitute

## Papers published from NSCI in 2014

#### Multisensory interface for 5D stem cell image volumes.

Koerner M, Wait E, Winter M, Bjornsson C, Kokovay E, Wang Y, Goderie SK, Temple S, Cohen AR. Conf Proc IEEE Eng Med Biol Soc. 2014 Aug;2014:1178-81. doi: 10.1109/ EMBC.2014.6943806.

## Interaction of amyloid inhibitor proteins with amyloid Beta peptides: insight from molecular dynamics simulations.

Das P, Kang SG, Temple S, Belfort G. PLoS One. 2014 Nov 25;9(11):e113041. doi: 10.1371/journal.pone.0113041. eCollection 2014.

# Visualization and correction of automated segmentation, tracking and lineaging from 5-D stem cell image sequences.

Wait E, Winter M, Bjornsson C, Kokovay E, Wang Y, Goderie S, Temple S, Cohen AR. BMC Bioinformatics. 2014 Oct 3;15:328. doi: 10.1186/1471-2105-15-328.

#### NPTX1 Regulates Neural Lineage Specification from Human Pluripotent Stem Cells

Boles NC, Hirsch SE, Le S, Corneo B, Najm F, Minotti AP, Wang Q, Lotz S, Tesar PT, Fasano CA. CELL Reports, 2014 6(4) 724-736.

# CORTECON: a temporal transcriptome analysis of in vitro human cerebral cortex development from human embryonic stem cells.

van de Leemput J, Boles NC, Kiehl TR, Corneo B, Lederman P, Menon V, Lee C, Martinez RA, Levi BP, Thompson CL, Yao S, Kaykas A, Temple S, Fasano CA.

Neuron. 2014 Jul 2;83(1):51-68. doi: 10.1016/j.neuron.2014.05.013.

#### A multiplex high-throughput gene expression assay to simultaneously detect disease and functional markers in induced pluripotent stem cell-derived retinal pigment epithelium.

Ferrer M, Corneo B, Davis J, Wan Q, Miyagishima KJ, King R, Maminishkis A, Marugan J, Sharma R, Shure M, Temple S, Miller S, Bharti K.

Stem Cells Transl Med. 2014 Aug;3(8):911-22. doi: 10.5966/sctm.2013-0192. Epub 2014 May 29.

#### Stem cells for retinal repair.

Stern J, Temple S. Dev Ophthalmol. 2014;53:70-80. doi: 10.1159/000357328. Epub 2014 Apr 10. Review.

#### Human RPE stem cells grown into polarized RPE monolayers on a polyester matrix are maintained after grafting into rabbit subretinal space.

Stanzel BV, Liu Z, Somboonthanakij S, Wongsawad W, Brinken R, Eter N, Corneo B, Holz FG, Temple S, Stern JH, Blenkinsop TA. Stem Cell Reports. 2014 Jan 2;2(1):64-77. doi: 10.1016/j.stemcr.2013.11.005. eCollection 2014 Jan 14



1 Discovery Drive Rensselaer, NY 12144





July 16, 2015 Wolferts Roost Country Club Join us for this meaningful event giving hope to those suffering with diseases of the central nervous system. Revolutionary treatments are on the horizon for life-altering diseases such as macular degeneration, spinal cord injury, Parkinson's disease and Alzheimer's. And cures are not far behind.

NSCI researchers led by world-renowned scientist Dr. Sally Temple will share the latest findings and discuss medical applications of stem cell research.

#### Be a stakeholder in a cure!

Entertainment by The Audiostars. Individual tickets \$125, table sponsor \$1,250.

For sponsorship information contact Suzanne Kawola (518) 694-8188, ext. 242.