This year, ISCI scientists made a revolutionary discovery: growth hormone-releasing hormone (GHRH), which is produced in the brain, can activate stem cells in the heart and repair damaged heart muscle.

That finding was made by the very scientist who discovered GHRH—Nobel Laureate Andrew V. Schally, Ph.D., M.D.h.c., D.Sc.h.c.—in collaboration with ISCI director Dr. Joshua Hare. Their work was published in the Proceedings of the National Academy of Sciences last January.

Dr. Schally received the Nobel Prize in Physiology and Medicine in 1977 for his discovery of hypothalamic hormones, which has not only benefited thousands of cancer patients worldwide, but laid the foundation for modern endocrinology. Dr. Schally first described GHRH in 1966, 11 years before receiving the Nobel. He continues to make similarly significant scientific breakthroughs to this day—the work on GHRH and heart stem cells being only one of many.

“This is a major step forward in harnessing a new therapeutic opportunity for heart disease that avoids unwanted side effects,” said Dr. Hare.

The scientists measured the impact of GHRH on the function and structure of rat hearts following a heart attack and found a dramatic improvement. The study also showed a reduction in the size of the area damaged by the heart attack as well as in cardiac fibrosis, one of the main biologic determinants of poor prognosis in heart failure.

“I hope that my work will benefit many patients. ISCI is a great site for this research because of its strong translational focus, which is only possible with outstanding physicians, such as Dr. Joshua Hare, a world-renowned cardiologist, to bring my discovery to cardiac patients,” said Dr. Schally.

Drs. Schally and Hare were awarded a two million dollar grant from the National Institutes of Health to continue their work.
Dear Friends,

Since publishing the first edition of Translation a few months ago, ISCI has continued to grow in size and stature.

We recruited new scientists to our faculty; launched clinical trials with sites at leading institutions such as Johns Hopkins; received more grant funding from the NIH; and published important findings in major scientific journals.

Not surprisingly, the physician-scientists at ISCI are garnering national and international recognition for their work. Together, they are striving to fulfill ISCI’s core mission: developing stem cell therapies for chronic, hard-to-treat diseases.

My research is focused on understanding the fundamental biology of survival and death, growth and regeneration in retinal neurons. Retinal degenerations often end with the death of retinal neurons, such as rod and cone photoreceptors in age-related macular degeneration and retinitis pigmentosa, and retinal ganglion cells in glaucoma. Although it may be possible to salvage these cells before they die, for example, using neurotrophic factors or gene therapy, for the many patients who have lost these cells, we must figure out a way to replace them. Little is known about how to harvest retinal stem cells, how to direct them to the proper location in the retina, how to push them to differentiate into retinal neurons, and how to encourage them to integrate into the retina. We’ve been studying how retinal stem cells differentiate into the retinal neurons that die in these diseases, with an eye towards replacing these cells. We’ve made progress in researching the survival and differentiation of human retinal stem cells in culture, and researching the differentiation conditions of human stem cells.

Our ultimate goal is to reverse blindness, or prevent it in patients with such diseases. The promise of stem cell science is two-fold: stem cells may support the survival and growth of sick or dying neurons in the retina, and they may replace those neurons already lost. A major goal for the lab is rapid translation: we want to take whatever we’ve learned in the lab and move it into the clinic as quickly and as safely as possible.

Dr. Joshua Hare
Director, ISCI

Want to support ISCI’s mission, enroll in a clinical trial or learn more about our research? Log on to www.med.miami.edu/isci/ or call 305-243-9106
In mid-August 2010, Dr. Joshua Hare’s clinical team began a key collaboration in cardiac regenerative medicine with The Johns Hopkins University in Baltimore, Maryland. Their work brought cutting edge medicine to patients whose heart attack caused permanent tissue damage due to a decrease in blood flow to the heart.

“ISCI’s alliance with The Johns Hopkins University creates the basis of a national network that facilitates future clinical trials in regenerative medicine,” said Dr. Hare.

The clinical trial, coined “POSEIDON” for Percutaneous Stem Cell Injection Delivery Effects on Neomyogenesis Pilot Study, tests the safety and efficacy of injecting 20 million, 100 million or 200 million mesenchymal stem cells inside the patient’s heart using a special cardiac catheter. The study also looks at the effects of using the patient’s own mesenchymal stem cells verses those of a donor, both of which are derived from adult bone marrow. ISCI’s cellular manufacturing laboratory, directed by Dr. Ian McNiece, produces the cells used in POSEIDON.

“Patients enrolled in the study have access to the research team 24 hours a day, 365 days a year,” said Darcy Velazquez, Research Nurse Manager. “This increased vigilance often results in better outcomes for the patient.”

The third patient cohort will begin in January 2011, where up to 10 patients with congestive heart failure will be injected with 200 million stem cells each to test the treatment’s capacity to regenerate heart tissue. This cohort is likely to bring in at least one additional study site at Rush University Medical Center in Chicago.

While Dr. Hare serves as the study’s principal investigator, Dr. Alan Heldman, an interventional cardiologist, trained his colleagues at Johns Hopkins on transendocardial injections. Both Drs. Hare and Heldman held faculty positions at The Johns Hopkins University prior to joining the Leonard M. Miller School of Medicine at the University of Miami. The study is funded by the National Heart, Lung and Blood Institute and is approved by the US Food and Drug Administration.

The Interdisciplinary Stem Cell Institute (ISCI) is committed to seeking philanthropic support to intensify basic and translational research across the disease continuum. Already in its third year of work, ISCI is honored to receive support from outstanding community leader and philanthropist H. Allan Shore, Esq., who serves on the University of Miami, Sylvester Comprehensive Cancer Center Board of Governors and the Office of Estate and Gift Planning Advisory Board.

Mr. Shore made a Major Gift in 2010 to establish a fund in memory of his parents, who suffered from heart disease. “The vision of the Shore Fund is to make a significant contribution to the mitigation or eradication of congestive heart failure,” said Mr. Shore.

This heartfelt gift has the capacity to impact the more than 600,000 people who die of heart disease in the United States every year. Congestive heart failure, a condition in which the heart can no longer pump enough blood to the rest of the body, too often takes the lives of those who suffered a heart attack within five years.

The Shore Fund will directly impact the translational research that ISCI Director Dr. Joshua Hare conducts. “Dr. Hare’s work, as well as his persona, is, aside from being brilliant, indelible in conveying the vision that stem cells hold as a solution to reversing damaged heart tissue. It is possible that we could actually see tangible results in our lifetime,” said Mr. Shore.

As ISCI continues to host like-minded scientist focused on the promise of stem cell therapies for chronic disease, bringing on board visionary philanthropic leaders will complete the synergy needed to propel basic research to a clinical setting.

“The team of lead ISCI scientists is so close to developing the next substantial therapy that can help so many. We remind ourselves each day that the patient is waiting,” said Dr. Hare. “The Shore Fund enables ISCI scientist to keep focused on finding the stem cell therapy that will prevent congestive heart failure from taking another life. I am ever so grateful for Mr. Shore’s support.”
The fourth episode of University of Miami’s series Breakthrough Medicine, Stem Cell Therapy: Healing Force of the Future, was awarded a 2010 Suncoast Regional Emmy in the category of Public/Current/Community Affairs.

The 24-minute documentary focuses on clinical trials conducted by ISCI scientists. To view the documentary, please visit www.uhealthsystem.com/breakthrough/


Jeffrey L. Goldberg, M.D., Ph.D., associate professor of ophthalmology, is the 2010 Hope for Vision scientist of the year. Dr. Goldberg is studying why neurons in the retina, especially retinal ganglion cells, fail to regenerate in degenerative diseases like glaucoma, or after an injury. Retinal ganglion cells transmit visual information from the retina’s photoreceptors to the brain, making them essential to sight. The cost of vision loss is extraordinary. More than $67 million is spent annually due to the direct and indirect costs of vision in our nation.

Karen Young, M.D., assistant professor of clinical pediatrics in the Division of Neonatology, is the 2010 recipient of the prestigious Micah Batchelor Award for Excellence in Children’s Health Research. Dr. Young, an ISCI investigator, plans to use the $300,000 grant that accompanies the award to fund research that aims to identify the factors that cause impaired development of lung cells in premature infants.

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Translation

Why call it Translation? Two reasons,
1. ISCI’s focus is translational medicine—taking basic scientific discoveries from the lab and “translating” them into clinical therapies for patients.  2. This publication aims to “translate” those discoveries and therapies, which are highly complex and technical, into language that is accessible to the non-scientist.

If you do not wish to receive further fundraising communications from the University of Miami’s Medical Programs, please send your request in writing to the University of Miami Office of HIPAA Privacy and Security, P.O. Box 019132 (M-879), Miami, Florida 33101.

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