Goldstein Foundation Gives $1.5 Million to Scripps Research

The Arlene & Arnold Goldstein Family Foundation has given $1.5 million to support research in The Scripps Research Institute’s Department of Molecular and Experimental Medicine.

The funds will make possible the hiring and support of an investigator designated the Arlene and Arnold Goldstein Assistant Professor of Molecular and Experimental Medicine who will contribute to the efforts to develop the next generation of drugs to treat transthyretin amyloidosis. Transthyretin amyloidosis can lead to a number of medical problems, including decreased heart function.

“We are very enthusiastic to partner with Arlene and Arnold Goldstein to develop the next generation of drugs to combat amyloidosis,” said Jeffery Kelly, chair of the Department of Molecular and Experimental Medicine, who is the Lita Annenberg Hazen Professor of Chemistry and a member of the Skaggs Institute for Chemical Biology at Scripps Research. “This category of human maladies represents a large unmet medical need and we appreciate the Goldsteins’ commitment to provide us with the resources to develop new therapeutic strategies for these degenerative disorders.”

Amyloidosis includes a number of inherited and acquired conditions caused by the misfolding of proteins in the body. In these diseases, microscopic fibrils made up of hundreds of misfolded proteins cluster together and deposit in organs, interfering with their normal function.

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Scripps Research Earns “Exceptional” Four-Star Rating for Eighth Straight Year!

In our current recession, it’s all the more important to have confidence that your donation is going where it is intended to go.

The Scripps Research Institute has again received a coveted four-star rating from Charity Navigator, the internet rating service that evaluates the efficiency and financial accountability of nonprofit groups. This is the eighth consecutive four-star rating for Scripps Research.

“Less than 1 percent of the charities we’ve rated have received at least eight consecutive four-star evaluations, indicating that The Scripps Research Institute outperforms most charities in America in its efforts to operate in the most fiscally responsible way,” wrote the organization’s president and chief executive officer, Ken Berger.

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The Kelly lab has contributed to efforts to understand the fundamental mechanisms of these diseases. In addition, the lab’s work has included the development of a compound that holds promise for treating transthyretin amyloidosis, which results from the misfolding of one of 100 mutants of the protein transthyretin. This compound is currently in a Phase II/III clinical trial by FoldRx Pharmaceuticals, a company founded by Kelly and colleagues.

The gift from the Goldstein Family Foundation will support work on the development of a distinct but complementary therapeutic aimed at treating senile systemic amyloidosis, a type of transthyretin amyloidosis that affects a quarter of the population 85 years of age or older.

This work will be a collaboration between the Kelly lab and the laboratory of R. Luke Wiseman, who will hold the newly created Arlene and Arnold Goldstein Assistant Professorship.

Wiseman will join Scripps Research this summer. “I am honored to accept the Arlene and Arnold Goldstein Assistant Professorship in Molecular and Experimental Medicine,” said Wiseman. “I look forward to beginning my career at Scripps Research and to developing innovative strategies to treat protein misfolding disorders.”

Wiseman, who received his Ph.D. from the Scripps Research Kellogg School of Science and Technology in 2005 and his undergraduate degree from the University of Virginia in 2001, is currently completing postdoctoral studies with Professor David Ron at the Skirball Institute at the New York University (NYU) School of Medicine.

Wiseman is the recipient of a National Research Service Awards Ruth L. Kirschstein Postdoctoral Research Fellowship, among other honors, and is the author of more than a dozen scientific publications.

Goldstein, whose foundation funded the new gift, grew up in the Bronx as the son of the founder of Atlantic Hardware & Supply Corp. A graduate of NYU’s School of Commerce, Accounts and Finance, Goldstein co-founded Samson Management, one of New York City’s largest owner management firms.

“I am a firm believer in ‘philanthropic capitalism,’” Goldstein said. “If one benefits from the fruits of capitalism, one should also give back to the community at large.”

A patient affected by amyloidosis, Goldstein is a participant in the ongoing FoldRx clinical trial. Support of medical research is not new to his foundation, however. In addition to a number of Jewish/Israeli causes, the Arlene and Arnold Goldstein Family Foundation has provided significant support to NYU Medical School, New York-Presbyterian Hospital, Columbia University Medical Center, Memorial Sloan-Kettering, and a number of other medical and research organizations.

“My reason for authorizing grants for medical research is that I feel this is an area that can help large segments of the world population,” Goldstein explained. Kelly is optimistic that this will be the case for Goldstein’s latest gift. “We are confident that we can discover three new classes of drug candidates,” he said, “and make a significant impact on protein folding disease.

Scripps Korea Antibody Institute

Scripps Research President Richard A. Lerner, Gangwon Province Governor Jin-Sun Kim, Chuncheon City Mayor Kwang-Jun Lee, and Gangwon National University President Young-Jung Kwon of South Korea recently signed an agreement to establish the Scripps Korea Antibody Institute on the Gangwon National University campus.

“I think the joint venture will help upgrade the research and development capability of South Korea and make Gangwon Province a regional bioengineering center,” said Governor Kim.

Four Stars, CONTINUED

And we have just learned that Charity Navigator ranks Scripps Research as number 10 among the Top Ten Charities with the Most Consecutive Four Star Ratings!

“This ‘exceptional’ rating from Charity Navigator differentiates The Scripps Research Institute from its peers and proves that it’s worthy of the public’s trust,” wrote Berger.

Organizations are evaluated on quantifiable criteria, including the ratio of fundraising costs to results and income growth.

“We’re delighted that Scripps Research is again being recognized as an outstanding organization,” said Wendy Scott Keeney, Vice President of Philanthropy at Scripps Research. “Gifts to the institute support excellence and innovation in research, both in basic science and in the prevention, diagnosis, and treatment of human disease.”
The Scripps Research Institute recently held its 17th commencement. The ceremony honored keynote speaker Stanford University chemist John Brauman and 41 students graduating from the Kellogg School of Science and Technology—the largest graduating class ever.

The graduate office was particularly busy in the weeks leading up to the commencement, ordering caps and gowns, checking the equipment, and welcoming friends and relatives of the graduating students as they arrived in San Diego from across the country and around the world, including from locations as far away as Korea and Nepal.

For the graduates, this momentous day was made possible not only by much individual effort, but also by the indispensable support of mentors, funding organizations, administrators, family, and friends.

A Ph.D. degree from the Kellogg School takes an average of five years, in which the candidate attends classes, completes lab rotations, and writes a dissertation that offers an original contribution to the field.

After welcoming remarks from Scripps Research President Richard A. Lerner and Kellogg School Dean James Williamson, Brauman congratulated the students, and addressed the topic of risks and rewards. He noted that the Scripps Research leadership took the risk of inventing the graduate program outside the standard paradigm, with exceptional results.

“This has provided a level of freedom and flexibility, and the opportunity to explore new avenues,” he said. “It has been amazingly successful and effective. It has not only changed science, but changed everyone’s vision. And everyone—education, research, and the general public—has gained…”

Brauman urged members of the graduating class not to shy away from taking risks in their own careers, because the results will ultimately be worthwhile.

Scripps Research alumni go on to hold positions in both academia and industry.

Employers of this year’s graduating class include: the National Institutes of Health; University of California, Berkeley; GE Global Research; Glaxo Smith Kline; National Comprehensive Cancer Network; Princeton University; Dana Farber Cancer Institute; University of Pennsylvania; University of California, Irvine; Gonzaga University; Stanford University; and Harvard University.

Congratulations, Class of 2009!

For the graduates, this momentous day was made possible not only by much individual effort, but also by the indispensable support of mentors, funding organizations, administrators, family, and friends.
 Scripps Research assistant professor Kevin Morris’s path as a scientist is a particularly unusual one. “For starters, I was a lousy student in high school, usually at the bottom of whatever class I was in, and I never gave science a thought,” said Kevin. But something happened in 1986 that changed Kevin’s life dramatically. That “something” was the headline-grabbing spread of HIV.

“My imagination was captivated by the idea that a virus—something you can’t see—can kill people. How did this killer operate? And what could we do to stop it?”

Kevin caught fire in his high school molecular biology class, going from worst to first. This sudden ascendancy was met with skepticism by his teacher. “On the mid-term, after I’d made the highest score, he said, ‘I think you cheated on this, but I just can’t figure out how,’” Kevin recalls, laughing.

As an undergraduate student focusing on wildlife and ecology at Humboldt State, he continued to be intrigued by the HIV virus, an intense interest that led him to the University of California, Davis, and a Ph.D. in 2001. Kevin did postdoctoral research in this field at the University of California, San Diego (UCSD), and then worked as a research scientist at the City of Hope before coming to Scripps Research in 2005 because of the high-powered science performed here.

Last year, Kevin was named as one of 30 “Tomorrow’s PIs (Principal Investigators)” by Genome Technology Magazine.

The investigators chosen to be Tomorrow’s PIs were selected for their innovative work and research in a discipline that’s part of the systems biology field.

Kevin’s research here is on RNA interference (RNAi). Also known as RNA silencing, RNAi is the introduction of double-stranded RNA into a cell to inhibit the expression of a gene associated with a disease. In our lab, we started asking whether we could use small interfering RNA to target gene promoters and turn the gene off or on at that level—transcriptional gene silencing,” said Kevin. “And we accomplished this.”

Not only did they accomplish it, but Kevin and his colleagues were able to turn the gene off after a two day treatment for up to 30 days. “As the silencing is epigenetic, it is very possible that it would be long-term and stable,” said Kevin.

“Over the past few years it has become increasingly apparent that many RNA-mediated modes of gene regulation are operative in biological systems,” Kevin explained. “Now scientists are beginning to understand just how pervasive this network is and to what extent it may be possible to apply this phenomenon for therapeutic benefit.”

Kevin describes the lab work as “solid but unspectacular science in action. It’s not dramatic—the dramatic aspect of the work is the questions we’re asking and the unique answers we’ve gotten.”

Kevin has shown the mechanism where small interfering RNAs when put into the cell, cause a specific upregulation in expression of the targeted gene. He and his colleagues discovered that the RNAs targeted a RNA-based suppressor, and that this action resulted in a loss of suppressor and turned up the specific gene.

“This was a really big discovery because the RNA-based suppressor was a non-coding RNA, which is thought to make up around 98 percent of the genome,” said Kevin elatedly. “We have been able to suggest some answers, with a few concrete examples, as to what the 98 percent of the genomes are doing.”

Kevin, whose lab counts only three people, including himself, was the first scientist internationally to publish evidence of this RNA activation phenomenon in human cells.

“I love the discovery aspect of my work,” said Kevin. “That ‘a-ha’ moment when everything comes together and fits is very rewarding.”

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“We are big believers in good community stewardship,” said Craig Grant, Regional President for National City’s Florida Market. “Our Grow up Great program is an unprecedented 10-year, $100 million investment in school readiness to help prepare underserved children up to five years of age for success in school and life. We also offer employees who volunteer 40 or more hours at an early education center a $1,000 grant to present to that center—this has sparked an extraordinary volunteer participation rate.”

“We believe in community and economic development — this is where our support of Scripps Florida falls, and we’re well regarded in this area; in fact, we won a 2007 Excellence in Corporate Philanthropy award from the Committee Encouraging Corporate Philanthropy and an award for corporate stewardship from the U.S. Chamber of Commerce.”

National City was a premier sponsor of the Scripps Florida opening ceremonies this year. Additionally, their Charitable Contributions Committee has awarded a postdoctoral fellowship to Robert Rounbehler, Ph.D., a Scripps Florida research associate in the Department of Cancer Biology, whose studies involve new drugs for cancer therapy. Plus noted Scripps Research stem cell scientist and Founding Director of the Center for Regenerative Medicine, Jeanne Loring, Ph.D., was the keynote speaker for one of National City’s Women in Power Luncheons that benefited the YWCA of Palm Beach County, with 130 in attendance. The series features highly successful women from all walks of life.

“Scripps Florida represents the future, and understands partnership,” said Kevin Sloan, President and Retail Market Executive of National City’s Southeast Florida Region. “Both National City and Scripps Florida are relatively new to Florida yet we’re totally committed to advancing the communities where we work, science, and the Florida economy. We look forward to continuing our partnership with Scripps in community outreach and funding for biomedical research and drug discovery.”

“The Palm Beach economy has traditionally been based on tourism and retirement,” said Mark Stevens, Executive Vice President and Managing Director of National City’s Private Client Group. “Scripps is bringing in a whole biomedical industry that is so good for our community in terms of economic vitality and diversity.”

National City purchased Fidelity Federal in 2006. Fidelity Federal, originally chartered in 1952 as West Palm Beach Federal Savings and Loan Association, was founded by three West Palm Beach Jaycees—attorneys Alan Brackett and William Cook, and George Preston, a mortgage broker and insurance agent. PNC acquired National City on December 31, 2008. PNC has 107 retail branches and seven wealth management offices in Florida, in addition to providing corporate and business banking.

As one of the largest corporate entities in every region of their footprint, National City is proudly committed to fulfilling its responsibility as a community partner—finding and sustaining positive solutions for community change.
**AWARDS AND HONORS**

**Scripps Research Wins Stem Cell Grants**

Scientists from The Scripps Research Institute have been awarded grants in the latest round of funding from the state stem cell agency California Institute for Regenerative Medicine (CIRM). In this round of grants—which supports projects to translate basic research into clinical cures—Scripps Research received more funds than any other institution.

The Scripps Research grants were made to:

- Professor Jeanne Loring, who was awarded $6.3 million for a project titled, “Ensuring the safety of cell therapy: a quality control pipeline for cell purification and validation.”
- Professor Martin Friedlander, who was awarded $5.9 million for a study titled, “Autologous Retinal Pigmented Epithelial Cells Derived from Induced Pluripotent Stem Cells for the Treatment of Atrophic Age Related Macular Degeneration.”

In addition, Darryl D’Lima, who is director of the Orthopaedic Research Laboratories at Scripps Health and an assistant professor at Scripps Research, will receive $3.1 million for a study titled, “Stem Cell-Based Therapy for Cartilage Regeneration and Osteoarthritis.”

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**Bruce Beutler Wins 2009 Albany Medical Center Prize**

Bruce Beutler, chair of the Department of Genetics at The Scripps Research Institute, has been named co-recipient of the prestigious Albany Medical Center Prize in Medicine and Biomedical Research, with Professor Ralph M. Steinman of The Rockefeller University and Professor Charles A. Dinarello of the University of Colorado. The trio was cited for groundbreaking discoveries that have transformed the field of immunology.

“Collectively, the work of these scientists has led to a dramatically better understanding of the human immune system, in health and in disease,” said James J. Barba, president and chief executive officer of Albany Medical Center, who served as chair of the National Selection Committee. “That knowledge has already directly resulted in new therapies for people with conditions including rheumatoid arthritis, diabetes, Crohn’s disease, and cancer. And, the discoveries they have made about how the body senses and responds to infection remain the basis of active research that holds the promise of new and improved vaccines and innovative ways to harness the power of the immune system to better fight viruses and bacterial illness. Their achievements are nothing short of astounding.”

The Albany Medical Center Prize was established in 2000 by the late Morris “Marty” Silverman to honor scientists whose work has translated from "the bench to the bedside" resulting in better outcomes for patients.

Professor Beutler is famous in scientific circles for defining the role of a key cytokine named Tumor Necrosis Factor (TNF). Specifically, Professor Beutler created recombinant inhibitors of TNF, now widely used as Enbrel, a first-line treatment for people suffering rheumatoid arthritis, psoriasis, ankylosing spondylitis, juvenile rheumatoid arthritis and other autoimmune disorders. The drug works by blocking TNF to reduce inflammation.

An immunologist and geneticist, Professor Beutler is a world leader in “forward genetics,” a technique used to identify critical genes supporting the innate immune system. His work has pointed to several genetic mutations in people who are predisposed to sepsis and other diseases.

To date 16 scientists have received the Albany Medical Center Prize. One of the world’s largest prizes for biomedical research, it includes a personal award of $500,000, to be shared equally among the co-recipients. Among the past prize winners, ten have also received the Lasker award. Three additionally received the Nobel prize.
Visit the Scripps Research Philanthropy Web Site!  
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On the site, you will find:

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- Information on Scripps Research’s mission, work, major scientific achievements, faculty and staff, graduate program, governance, budget, and public programs
- Abstracts on various Scripps Research disease breakthroughs, categorized by disease
- Information on upcoming lectures and receptions
- Personal stories of our scientists, their work, and what drives them
- Profiles of our donors and why they give to Scripps Research
- Opportunities for giving
- A section on planned giving opportunities in which you and your family can receive substantial tax benefits while assisting Scripps Research
- Accountability information, such as our Form 990 and Charity Navigator ranking
- The ability to make credit card contributions via our secure payment processor
- The ability to make tribute gifts in memory or honor of a loved one and to forward the tribute to friends, family members and colleagues
- The ability to become a monthly donor with your gift automatically transferred from your debit or credit card each month
- Archived editions of Scripps Discovers, our donor newsletter
- Information on our community activities
- Contact information for philanthropy department staff to better serve you
- Scripps Research banners that you can place on your own web site to help generate awareness and fundraise for the institute

Please take a few minutes to visit our site and sign up for the e-newsletter!

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Kevin Morris, CONTINUED

The potential therapeutic benefits are enormous, according to Kevin, and he is now working to commercialize his approaches to treat and silence HIV.

“If you can find a gene that’s specifically linked to a particular disease through this process you can theoretically turn the gene off.” So far in his lab, genes of interest have been “turned down” by as much as 80 percent. “The main point is we’ve shown how to manipulate a gene’s function and power.”

Kevin’s specific interest is in utilizing RNA interference to treat HIV, and this research is also leading him to a better understanding of the way that pancreatic cancer grows and develops, and the challenge of controlling genes associated with tumors.

“I really wanted to get involved with pancreatic cancer because it is so deadly—it has only a 5 percent survival rate over five years,” said Kevin. “I believe targeting genes and being able to turn them on and off can stop the cancer from metastasizing.”

“I am very excited about what we do,” said Kevin. “We can specifically target any gene we want. I believe we can pick just about any disease to attack.”

“I’m not very smart—I can’t remember things,” concluded Kevin. “But I think I’m pretty creative and not confined by subjectivity—I try experiments that others won’t in order to push the avenues of science further.”
Scripps Research continued its Frontiers in Science lecture series for donors and friends in March at the Estancia La Jolla Hotel and Spa. At the event, Scripps Research professor Nicholas Schork spoke on “Personalized Medicine in the Era of Genomics—Changing the Future.” Over 200 Scripps Research contributors and friends heard about how studying genetics and genomics, the blueprints for human life, will help predict and determine an individual’s risk for getting diseases, such as cancer, diabetes, Alzheimer’s, Parkinson’s, and cardiovascular disease. Professor Schork illustrated how genetics and genomics are leading the way to personalized medicine, a field whose goals are to optimize preventive healthcare strategies and drug therapies while people are still well or at the earliest stage of disease. Pictured at the reception are Scripps Research supporters Paul and Carol Webb.

Alex Dreyfous, Scripps Research trustee, and a major supporter of Scripps Florida, and his wife Renate brought their yacht, the “Silver Cloud” to San Diego, in March, and held an onboard reception for 35 Scripps Research supporters and friends. Richard A. Lerner, M.D., president of Scripps Research, provided remarks. Pictured at the event are Alex and Renate Dreyfous.

Deborah Leach-Scampavia (left), Scripps Florida science education administrator, receives a donation from the Admiral’s Cove Cares Charitable Foundation for support of honorariums for Scripps Research graduate student presentations in K-12 science classrooms. Also pictured (l to r) are Lisa Emalfarb and Win Gerson, both of Admiral’s Cove, and Barbara Suflas Noble, director of external affairs at Scripps Florida.

thinkPINK kids of Wellington High School, Florida, present a check for $7,000 to John Cleveland, Ph.D., Chairman of The Scripps Research Department of Cancer Biology. The monies were raised via a 5k “Walk to Win the Battle Against Breast Cancer” at their school. It was an evening filled with entertainment, food and fun to help raise money for breast cancer research. Pictured with Professor Cleveland are the “kids,” (l to r) Benjamin Aqua, Eliza Schlein, Lindsay Rosenthal, and Josh Hyber.