



Florida Tomorrow

Evelyn F. and William L.
McKnight Brain Institute



UF | FLORIDA
TOMORROW
THE CAMPAIGN FOR THE UNIVERSITY OF FLORIDA



From the Director

It is a special time in the brain sciences.

My experience as executive director of the Evelyn F. and William L. McKnight Brain Institute over the past several years has reinforced what I have learned in more than three decades as a research scientist and teacher: We are making great discoveries that will reshape the future of health care, and the need to put these discoveries to practical use grows more urgent by the day.

Exciting new findings — including groundbreaking discoveries made at UF — tell us that the brain can repair itself after injury or disease, but it needs assistance from a new field called “regenerative medicine.”

A decade ago, scientists thought people were born with a fixed amount of irreplaceable brain cells. We now know our brains produce cells and connections throughout adulthood. Furthermore, we have shown ordinary human brain cells may share the prized qualities of self-renewal and adaptability associated with stem cells.

Unprecedented hope now exists to develop treatments for brain diseases, and the public shares this hope. Confidence in biomedical research has never been stronger.

A major thrust of our efforts is to encourage neurogenesis — the generation of brain cells — and to find ways to protect existing cells.

By focusing on adult stem cells and exploring causes of disease, we aim to develop therapies to fight ailments such as Alzheimer’s disease, Parkinson’s disease, multiple sclerosis, epilepsy, stroke, brain cancer, age-related memory loss and Huntington’s disease.

Undoubtedly, the mission is challenging. But the university is equal to the task. For the sake of an aging population of Americans, we must succeed. It will take expertise in the biological sciences, tissue engineering and clinical practice to accomplish our goals. It will take strategic investment in scientists, clinicians and facilities. In addition, the financial support of friends, partners and donors from the Gator Nation and beyond is needed to augment the funding our scientists earn through National Institutes of Health and other competitive grants.

The reward will come when people are protected from Parkinson’s, Alzheimer’s, brain cancer and a host of other neurological diseases, and when our own cells can be mustered to fight devastating neurological problems.

That is the McKnight Brain Institute’s vision for *Florida Tomorrow*. Please, share it with us.

Sincerely,

Dennis A. Steindler, Ph.D.

Executive Director, McKnight Brain Institute of the University of Florida

Florida Tomorrow

... and the Evelyn F. and William L. McKnight Brain Institute

The Promise of Tomorrow

The University of Florida holds the promise of the future: *Florida Tomorrow* — a place, a belief, a day. *Florida Tomorrow* is filled with possibilities. *Florida Tomorrow* is for dreamers and doers, for optimists and pragmatists, for scholars and entrepreneurs, all of whom are nurtured at Florida's flagship university: the University of Florida, the foundation of the Gator Nation.

What is *Florida Tomorrow*? Here at the McKnight Brain Institute, we believe it's an opportunity, one filled with promise and hope. It's that belief that feeds the university's capital campaign to raise more than \$1 billion.

The *Florida Tomorrow* campaign will shape the university, certainly. But its ripple effect will also touch the state of Florida, the nation and the entire world. *Florida Tomorrow* is pioneering research and spirited academic programs. It's a fertile environment for inquiry, teaching and learning. It's being at the forefront to address the challenges facing all of us, both today and tomorrow.

Evelyn F. and William L. McKnight Brain Institute *Florida Tomorrow Campaign Goal*

Program Support and Research

\$25 million



Dr. Kelly Foote



UF physicians
Michael Okun,
Hubert Fernandez,
Kelly Foote and
Ramon Rodriguez.



Florida Tomorrow is a place ...

where the best researchers and clinicians in the world are rallying to defeat even the most daunting brain disorders.

To Tame an Epidemic

They may not be the diseases that grab headlines, but movement disorders such as Parkinson's are an epidemic, says Dr. Michael Okun.

"These disorders aren't infectious, so people don't get scared like they do about something like bird flu," he notes. "But the reality is, they affect many more people worldwide than any of the infectious diseases you hear about on the nightly news."

Okun, along with Drs. Kelly Foote and Hubert Fernandez, co-directs UF's Movement Disorders Center at the McKnight Brain Institute. As the population ages, he says, the burden created by movement disorders is increasing.

In answer to the growing need, the McKnight Brain Institute is forging an extraordinary center where patients have access to the latest clinical trials — 25 are already in progress — as well as to leading scientists investigating gene therapies, regenerative techniques and drug treatments.

Scientists are optimistic. Using a commonly occurring virus to insert corrective genes, MBI researchers have revitalized brain cells and restored normal movements in primates with a drug-induced form of Parkinson's disease. Similarly, they've used gene therapy to completely reverse abnormal movements called

dyskinesias in laboratory rats, which is promising for patients struggling with the flailing movements that occur during conventional drug treatments for Parkinson's disease.

The next step in the evolution of therapies may involve the introduction of genetically engineered stem cells into the brain using the delivery viruses pioneered at UF. Stem cells, whether they already live in our bodies or come from donors, have a natural ability to target and repair an injured site.

"The home run is a drug or combination of drugs that will, as any cell starts to die in your brain, activate your own indigenous stem cell population," says Dennis Steindler, executive director of the McKnight Brain Institute.

With molecular biologists researching movement disorders within arm's length of a patient clinic and drug developers, anything is possible. Tremendous strides have already been made.

"We see children who are suffering so much they have been given up for lost, and after treatment they are able to get up out of a wheelchair and lead a much more normal life," Okun says. "The amazing things you hear about happening very rarely happen with some frequency around here."



A major thrust of the project compares stem cells from simple systems in invertebrates and non-mammalian vertebrates with adult human stem cells.

Florida Tomorrow is a day ...

when people's own regenerative cells can be mobilized to treat and cure whatever medical problems are ailing them.

Secrets of Regeneration

Scientists could learn a lot from “the squishies.”

That’s the nickname Dr. Edward Scott uses to refer to creatures — from starfish to salamanders — with remarkable regenerative capabilities.

“We’re studying creatures that, if their spinal cords are cut, will heal themselves and walk again,” says Scott, a professor in the department of Molecular Genetics and Microbiology and the director of the MBI’s program of Stem Cell Biology and Regenerative Medicine. “We don’t regenerate nearly as well.”

The Regeneration Project, headed by Scott and MBI Executive Director Dennis Steindler, seeks to unlock the secrets behind these animals’ healing abilities and to use those findings in human therapies.

“If a child loses a fingertip, it will grow back,” Scott explains. “After puberty, an injury like that is less likely to regenerate; and if it happened as an adult, it wouldn’t re-grow at all. But the fact that a child can re-grow a fingertip suggests that some of those pathways are probably still there.”

A major thrust of the effort compares stem cells from simple systems in invertebrates and non-mammalian vertebrates with normal and diseased adult human stem cells.

MBI researchers have already discovered 100 genes in a sea slug called *Aplysia* — an important laboratory model of learn-

ing and memory — similar to genes in humans associated with all major neurological diseases, as well as more than 600 similar genes that control development.

Scott hopes the project’s research will lead to better treatments for spinal cord injuries and diseases from Alzheimer’s to macular degeneration.

“Once we find specific instances where we don’t regenerate but these species do, we can find out what the differences are and look at how to overcome them,” he says.

Through annual summits and fellowships, the project will bring together physicians, medical researchers, marine scientists and biomedical engineers to uncover the mysteries of regeneration and develop treatments based on their discoveries. Scott sees promise in the project’s multidisciplinary approach.

“We have marine scientists who may be working with a materials science engineer and a neurologist who’s helping a patient recover from a stroke. These are people who normally would never be in the same room together,” he says.

“In the next year and a half, I’m hoping to see our research used in three different clinical trials. Seeing people helped by research is what keeps me going.”

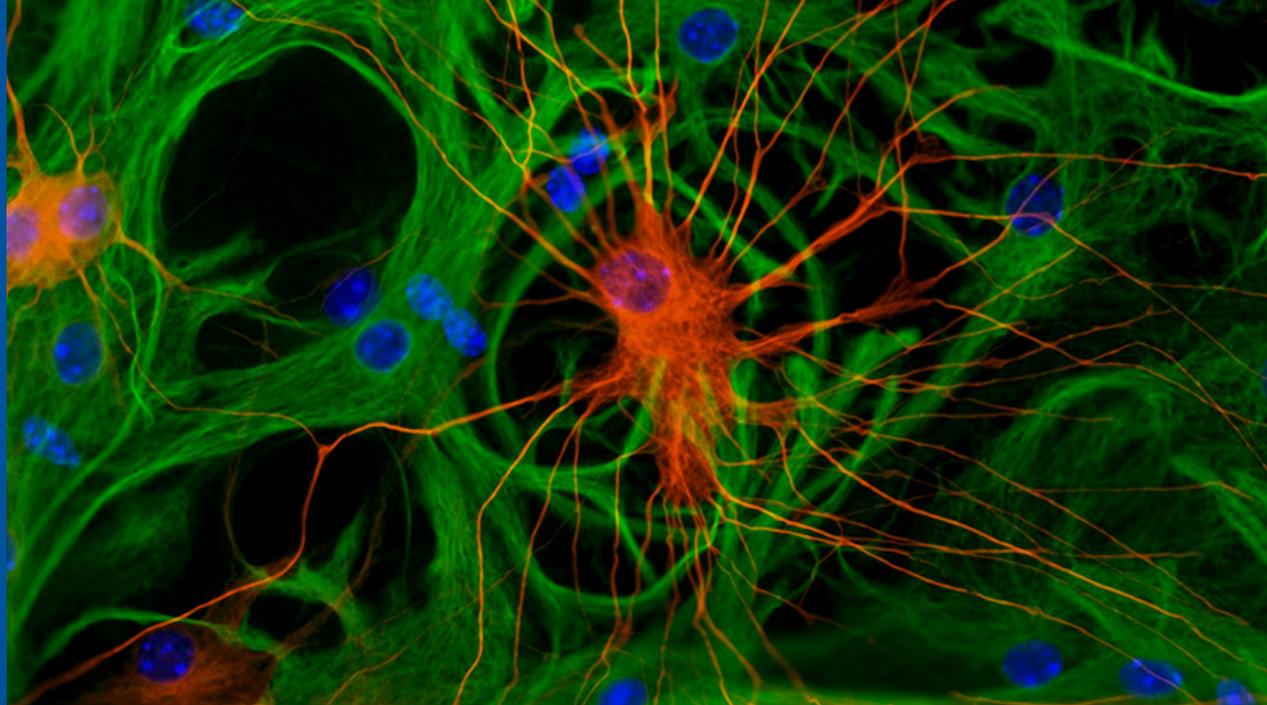


Dr. Edward Scott



Dr. William Friedman

Understanding how normal brain cells and brain tumor cells are generated will enhance efforts to treat cancer.



Florida Tomorrow is a belief ...

that nothing is impossible in terms of curing neurological diseases.

Battling Brain Cancer

Dr. William Friedman has always been dedicated to helping patients fight the devastating effects of brain tumors. But when his mother died of a brain tumor in 2003, he redoubled his efforts.

“Until that point, I had been focused on ways to give the very best care to one patient at a time,” says Friedman, chairman of neurosurgery at UF’s McKnight Brain Institute. “I realized I wanted to try to do something that could result in a breakthrough for hundreds of thousands of brain tumor patients.”

Today, Friedman, along with MBI Executive Director Dennis Steindler, helps oversee a statewide tissue bank of brain tumor samples — the most comprehensive of its kind in the country. UF scientists believe the tissue, collected from patients at UF and four sister institutions, will provide data for researchers searching for treatments and cures.

“The major obstacle in brain tumor research is similar to the major obstacle in other cancers: We don’t have a clear understanding of the molecular basis of the tumors,” Friedman explains. “Understanding that puzzle will lead to the discovery of much more effective treatments and cures. This is an opportunity to do the molecular genetic research we believe will lead to the next big breakthrough.”

Groundbreaking discoveries have already been made. MBI regenerative medicine scientists were the first to identify a cancer

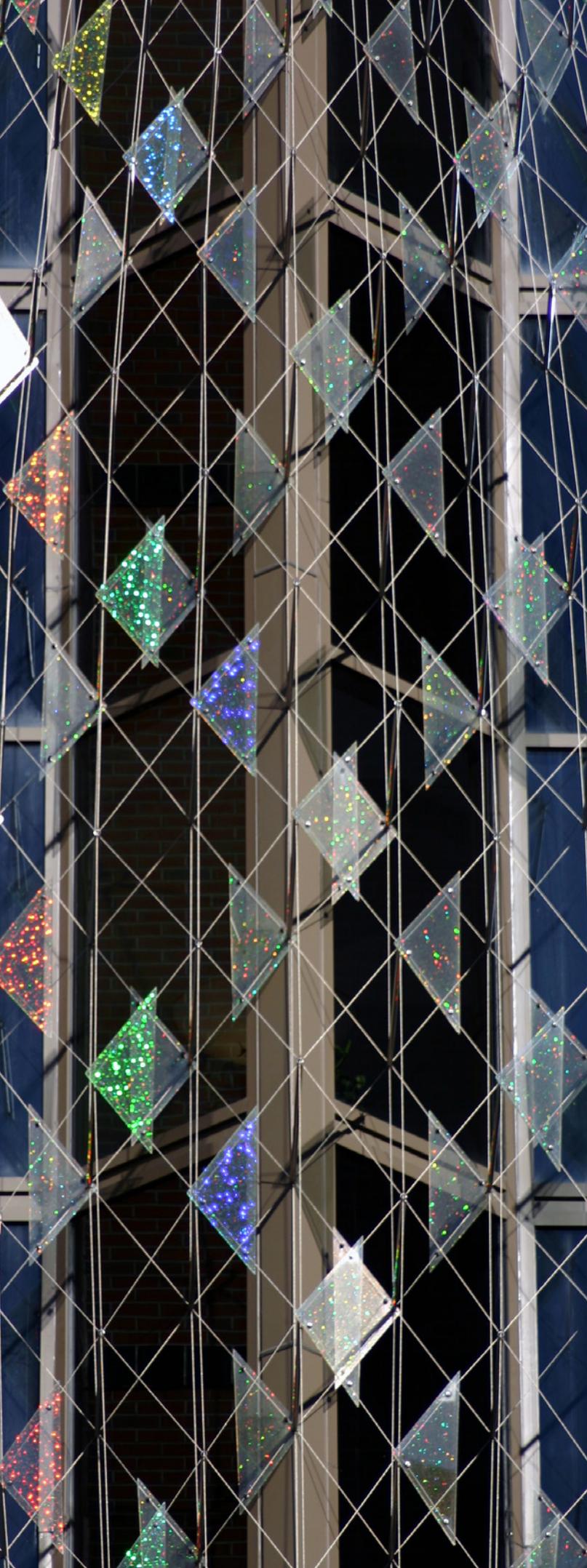
stem cell population within human brain tumors, drastically shifting the scientific world’s understanding of brain tumor growth. Further analyses of cancer stem cells promise to reveal how normal brain cells and brain tumor cells are generated.

About 190,000 Americans are diagnosed with brain tumors each year, most frequently in children and older adults. Primary malignant tumors — those that begin and tend to stay in the brain — are particularly devastating because of their alarmingly rapid assault. Many patients die within a year of diagnosis, some within a few months.

Support recently arrived in the form of a \$5 million gift from the Fort Lauderdale-based Lillian S. Wells Foundation Inc. to the Brain Institute’s brain tumor therapy center — now known as the Preston A. Wells Jr. Center for Brain Tumor Therapy. The contribution reinforces efforts to recruit world-class doctors and scientists and test experimental therapies. Eight investigational treatment protocols are under way.

“Our patients are coming to us when they are most in need of help, support, someone who cares about them,” Friedman says.

As someone who has experienced that heartache firsthand, he can empathize. But when he and colleagues at the McKnight Brain Institute look to the future of brain-tumor research at UF, they can offer something more: hope.



Our Vision of Tomorrow

Scientists at the Evelyn F. and William L. McKnight Brain Institute of the University of Florida are committed to winning the battle against brain diseases and injuries.

Already our clinicians and scientists have helped patients challenged by Parkinson's disease and other movement disorders resume more normal lives. We've helped cancer patients maintain their quality of life, and we've provided newfound understanding for doctors treating patients who are addicted to prescription or illicit drugs. We are working to undo the ravages of Alzheimer's disease and seek ways to keep older minds sharp well into what are traditionally considered to be "the senior years."

We are optimistic. Never before has science been so close to tapping a person's own natural, reparative abilities to combat the effects of Alzheimer's disease, Parkinson's disease, multiple sclerosis, epilepsy, brain cancer, age-related memory loss and a host of neurodegenerative diseases. Conversely, never before has the enormity of the task been in such stark focus.

The studies necessary to reveal the fundamental causes of disease and the translational research to develop the innovative therapies of tomorrow require mastery in fields that stretch across the biological sciences, medicine and engineering. We bring state-of-the-art laboratory facilities to the task, as well as federally funded clinicians and scientists with world-class expertise in regenerative medicine, gene therapy, brain imaging, biomedical engineering, neurosurgery, addiction medicine and drug development.

(right) Dr. Michael Okun



But to make an even greater impact on patients with neurological problems, we need to attract even more leading scientists and ideas, and integrate them into the high-powered discovery machine that is the McKnight Brain Institute. That's why it is vital to receive support from the Gator Nation and anyone whose life may have been touched by a friend or loved one with a neurological disorder. We have begun major initiatives in brain research for age-related memory loss, Alzheimer's disease, movement disorders, stroke and chronic pain. The scope of the projects at the McKnight Brain Institute is truly amazing.

We envision a \$60 million expansion of the McKnight Brain Institute facility to build a "supercenter" to study and treat movement disorders, including Parkinson's disease and dystonia. We are creating a place where people with neurological

problems come to receive the best, research-guided patient care in the world.

We are assembling the top minds from UF and the world for "The Regeneration Project," which will connect scientists who work with adult human stem cells — the building blocks that exist within our brain, bone marrow and blood — with scientists who study how tissues and limbs develop in a variety of organisms. The payoff will come when we find ways to turn on long-silent genes to repair or replace lost organs, or find ways to mobilize and reinforce the body's own supply of adult stem cells to protect against or fight neurodegenerative diseases.

We are fighting brain cancer. New brain tumors arise in more than 200,000 Americans each year. People in their 60s face the highest risk each year. The Florida Center for Brain Tumor Research at the Brain Institute is working with hospitals statewide to collect tissue taken during brain surgeries and share data on brain tumors. These tumor specimens represent a treasure trove for basic and clinical scientists who are conducting studies at the cellular and molecular level to understand how brain tumors develop and grow, and how we might be able to use this knowledge to disarm or even destroy them.

Gifts and pledges to the McKnight Brain Institute strengthen our research, patient-care and educational activities. They may be designated for endowments that support researchers, clinicians or students, or to support equipment and facilities. Many gifts are eligible for matching support from the state of Florida.

With your help, the McKnight Brain Institute of the University of Florida will change the world.





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University of Florida Foundation, Inc. | P.O. Box 14425 | Gainesville, FL 32604 | (352) 392-1691 | www.FloridaTomorrow.ufl.edu