

TOWARD  
A WORLD  
OF HEALTHY  
CHILDREN

# Research Highlights 2007

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The Saban Research Institute  
of Childrens Hospital Los Angeles

01	Note from the Director	1
02	Overview of the Year	3
03	Research Programs and Initiatives	4
04	Clinical Research Centers	13
05	Research Education	16
06	Awards and News	17
07	Honor Roll of Donors	18
08	Investigators	20
09	The Saban Research Institute Committee	21

*The microscopic and cellular images seen in this publication were produced by investigators in The Saban Research Institute of Childrens Hospital Los Angeles.*

**The Saban Research Institute**



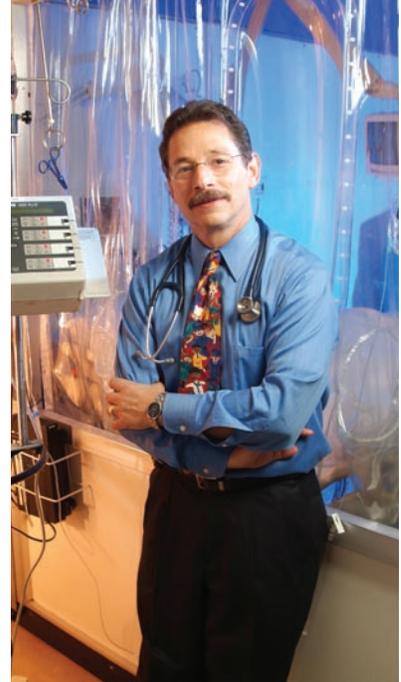
**ChildrensHospitalLosAngeles**

*International Leader in Pediatrics*

**KECK**  
SCHOOL OF MEDICINE OF USC

01

## NOTE FROM THE DIRECTOR



### Donald B. Kohn, MD\*

**Acting Director, The Saban Research Institute**  
**Director, Gene, Immune and Stem Cell Therapy Program**  
**Co-Director, Childrens Center for Cancer and Blood Diseases**

This past year, I have had the privilege to serve as Acting Director of The Saban Research Institute while Director Yves DeClerck, MD\*, has performed a research sabbatical. This position has given me new insights into the extraordinary successes of our dedicated investigators and interdisciplinary programs.

The 106-year history of Childrens Hospital Los Angeles centers on kids. This is the basis of our research, which sets us apart from other freestanding biomedical research institutions in Southern California and gives our investigations unique value.

We approach our pediatric studies differently than adult-based research, but we are always aware this work may have adult

applications. We are very good at what we do, and The Saban Research Institute is the largest recipient of National Institutes of Health funding for pediatric research on the West Coast. However, federal funding recently has suffered significant declines, which could pose a threat to our work at the research forefront, since 88 percent of our funding comes from these sources.

Our plans remain ambitious even though we will face unique challenges to fulfill our vision of a world without pediatric disease. As always, our investigators are grateful to our philanthropic partners. Your support has never been more important to The Saban Research Institute and the children we serve; together, we are making a world of difference.





## Toward a World of Healthy Children

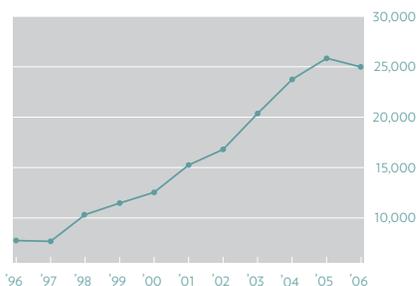
Investigators at The Saban Research Institute of Childrens Hospital Los Angeles are determined to create a world in which all children are healthy—where they are no longer threatened by such diseases as cancer, congenital heart defects, diabetes, sickle cell anemia, epilepsy, autism, immune deficiencies and respiratory disorders. This is one of the country's few freestanding research centers to combine scientific inquiry with clinical care—devoted *exclusively* to children.

In 2007, our researchers and physician-scientists sought to ask basic questions about human biology; see inside the body in new ways; explore the enormous potential of stem cells; unlock the mysteries of cancer cells; understand the basis for birth defects; discover influential genes; and develop new tools to prevent and treat serious illness in children in the hospital and in the community. In addition, we dedicated new resources to clinical research and expanded our innovative program of research education.

In this edition of *Research Highlights*, you will read about some of our most recent scientific achievements. They represent a small percentage of the work under way here at The Saban Research Institute, where 93 investigators are engaged in 200 laboratory studies, clinical trials and community-based research and health programs. For the ninth consecutive year, The Saban Research Institute ranks among the nation's top five stand-alone pediatric research facilities in funding from the National Institutes of Health (NIH), and it is the pediatric funding leader in the western United States.

### National Institutes of Health (NIH) Funding 1995-2006

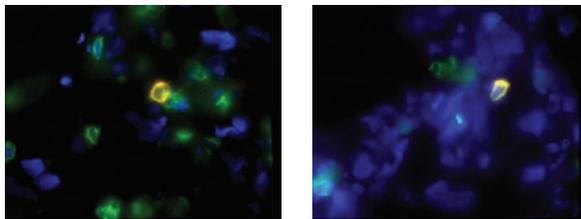
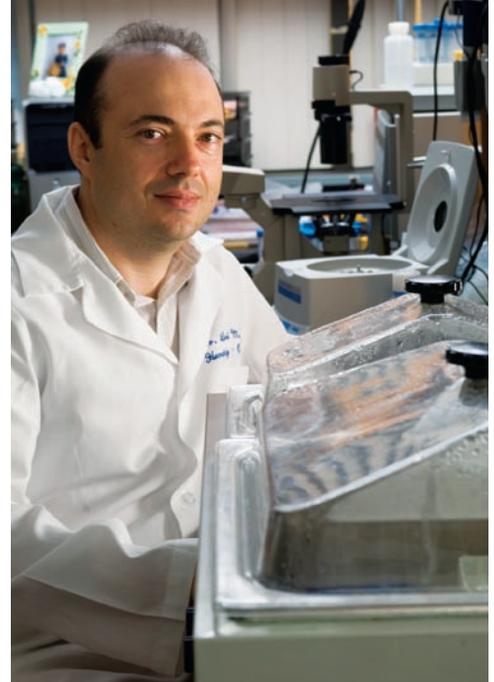
For biomedical research at  
The Saban Research Institute



*Despite a significant decrease in NIH funding across the nation, Childrens Hospital Los Angeles received \$25 million in funding from the NIH in Fiscal Year 2006, which represents a modest decrease of 2.5 percent from the previous year's \$25.6 million. Such grants are the result of a rigorous peer-review process, reflecting the national excellence and promise of our investigations.*

# RESEARCH PROGRAMS

Leonid Metelitsa, MD, PhD



Left: Natural killer T cells (yellow) among tumor cells (blue) and other tumor-infiltrating T cells (green), in cells from neuroblastoma

## Cancer Program

Director: **Robert C. Seeger, MD\***  
[www.childrenshospitala.org/research/cancer](http://www.childrenshospitala.org/research/cancer)

When it comes to treating deadly pediatric cancers, physicians too often see youngsters who have reached their limit for curative therapy—their bodies simply cannot tolerate further chemotherapy or radiation. But due to the work of researchers such as Leonid Metelitsa, MD, PhD\*, an exciting alternative is emerging: immunotherapy—arming a patient’s own immune system to fight cancer from within.

Three years ago, Dr. Metelitsa and Robert C. Seeger, MD\*, identified the cancer-fighting power of a subset of white blood cells called “invariant natural killer T”

(NKT) cells. They discovered that these cells infiltrate the tumors of only one-third of all children diagnosed with neuroblastoma and that these youngsters enjoyed a far better prognosis.

Dr. Metelitsa’s laboratory is the only one in the United States to study NKT cells in pediatric disease. In 2007, again in collaboration with Dr. Seeger, he clarified the mechanism that controls NKT cell migration to the tumor site, pinpointing a strong inverse association between their presence and expression of MYCN, a cancer gene that transforms normal cells into malignant ones. He also learned that NKT cells reside most abundantly in tumors that exhibit high levels of the protein CCL2/MCP-1.

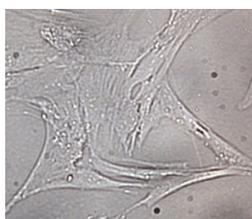
Like a beacon, CCL2 attracts NKT cells, which in turn, mediate immune attack against cancer. Findings from these investigations were published this summer in the prestigious *Journal of Clinical Investigation*. Next, Dr. Metelitsa will combine novel agents, which stimulate NKT cells, with an experimental cancer vaccine designed to provoke a patient’s own immune system to recognize and selectively attack tumor cells. “Another goal of the vaccine is to form immunological memory, so that if any cancer cell survives and later attempts to proliferate, the patient’s immune system will control it,” he says.

Potentially, this work could produce a wide array of therapies for pediatric cancers, as well as those affecting adults.

\* Faculty member, the Keck School of Medicine of the University of Southern California



Mirja Gunthart, PhD, research specialist (left), with Mary Kearns-Jonker, PhD



Far left: Model of human heart.  
Left: Genetically modified fibroblasts (connective tissue cells) from fetal pigs shown with endothelial cells (cells lining the heart or blood vessels), used in studies on the immune response to xenografts

## Cardiovascular Research Program

Director: **Vaughn A. Starnes, MD\***  
[www.childrenshospitala.org/cardiovascular](http://www.childrenshospitala.org/cardiovascular)

At any given time, more than 200 children in the United States are waiting for a heart transplant in order to live, according to the United Network for Organ Sharing. But many of these children will die waiting for a donor organ—prompting scientists to explore the possible use of xenografts— hearts from animals such as pigs that might help sustain patients until human organs become available.

Less than a decade ago, xenografts were rapidly rejected by recipients' antibodies within hours of transplantation. More recently, researchers have learned to genetically modify the donor animals, and

rejection time has been extended to as long as six months. The question is: can that period be stretched further, even long enough to make the technique practical?

Mary Kearns-Jonker, PhD\*, at The Saban Research Institute is tackling this question in two ways. First, she conducts research to understand the immune response that is responsible for xenograft rejection. Investigators elsewhere have “knocked out” expression of the Gal carbohydrate on donor organs, which would otherwise initiate rejection. “We hypothesize that there’s an alternative carbohydrate that initiates rejection when the Gal carbohydrate gets eliminated,” says Dr. Kearns-Jonker.

Her team’s goal: to zero in on that target, understand how humans respond to it and

use that knowledge to improve xenograft engineering.

Dr. Kearns-Jonker also is examining techniques that induce tolerance to xenografts. In an ongoing mouse-model study, she has introduced into the bone marrow a gene that encourages its immune system to accept xenograft cells. The results are promising: mice receiving genetically modified bone marrow permanently accept heart grafts.

If techniques like these can be successfully applied in humans, then xenografts might serve as more than temporary solutions to organ need—they may become semi-permanent transplants, sustaining recipients for many years.

Michele D. Kipke, PhD



*Above: cross-section model of the human brain*

**“We cannot afford to deliver anything but the most effective interventions to children with autism spectrum disorders.”**

-Michele Kipke, PhD

## Community, Health Outcomes and Intervention Research Program

Director: **Michele D. Kipke, PhD\***  
[www.childrenshospitala.org/community](http://www.childrenshospitala.org/community)

Over the past decade, diagnoses of autism among America’s children have increased dramatically. The federal Centers for Disease Control and Prevention estimate that this developmental disorder of brain function impacts one in 150 births in the United States, making it more common than pediatric cancer, diabetes and AIDS combined. Yet, there is no effective prevention, treatment or cure.

Michele Kipke, PhD, is determined to change that reality, armed with the Las Madrinas Endowment for Autism Research, Interventions and Outcomes, which was awarded in 2007.

“New research has demonstrated that early diagnosis and intervention are critically important to gaining maximum benefit from existing therapies for autism spectrum disorders (ASD),” says Dr. Kipke. However, research conducted to date has not rigorously evaluated and compared comprehensive interventions for their effectiveness.

The \$5 million Las Madrinas Endowment for Autism Research, Interventions and Outcomes will fund development of a diagnostic and intervention research laboratory at Childrens Hospital Los Angeles, in which tools and technologies will be used for early diagnosis of ASD, and new intervention research will be initiated to evaluate what works best for whom and why. This is the latest, ambi-

tious fund-raising goal for Las Madrinas, a community-based group that has supported Childrens Hospital since 1933.

On the agenda in the year ahead is the recruitment of nationally recognized scientists with expertise in early diagnosis and treatment. Over time, Dr. Kipke would like to see the development of tools for screening for ASD in community settings, such as pediatricians’ offices, clinics and schools.

A high priority will be placed on tracking both short- and long-term outcomes. “We cannot afford to deliver anything but the most effective interventions to children with ASD,” says Dr. Kipke. “We know we can make many important contributions to the lives of so many children and families.”

\* Faculty member, the Keck School of Medicine of the University of Southern California

Saverio Bellusci, PhD



**“FGF 10 is pivotal not only in embryonic liver development, but in ongoing liver function. This suggests potentially exciting clinical applications.”**

-Kasper Wang, MD

## Developmental Biology Program

Director: **David Warburton, DSc, MD, FRCP\***  
[www.childrenshospitala.org/devbiol](http://www.childrenshospitala.org/devbiol)

When clinical surgery comes together with basic research, the energetic collaboration can result in new ideas for the children who need them. A case in point: Kasper S. Wang, MD\*, a surgeon in the Division of General Pediatric Surgery at Childrens Hospital Los Angeles, and Saverio Bellusci, PhD\*, an investigator in The Saban Research Institute. Together, they are exploring the mysterious ways in which a natural protein called Fibroblast Growth Factor 10 (FGF 10) acts on the liver.

As a pediatric surgeon, Dr. Wang needs his patients to have a healthy liver because without it, their blood can't clot, toxins

accumulate and essential proteins dissipate. “It turns out FGF 10 is pivotal not only in embryonic liver development, as we expected, but in ongoing liver function,” says Dr. Wang, who conceived the investigation and serves as principal investigator.

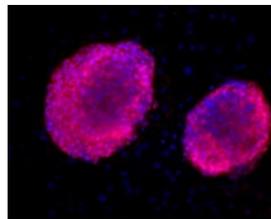
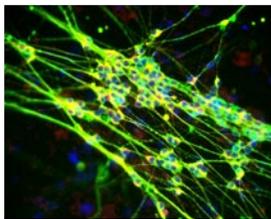
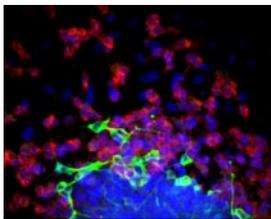
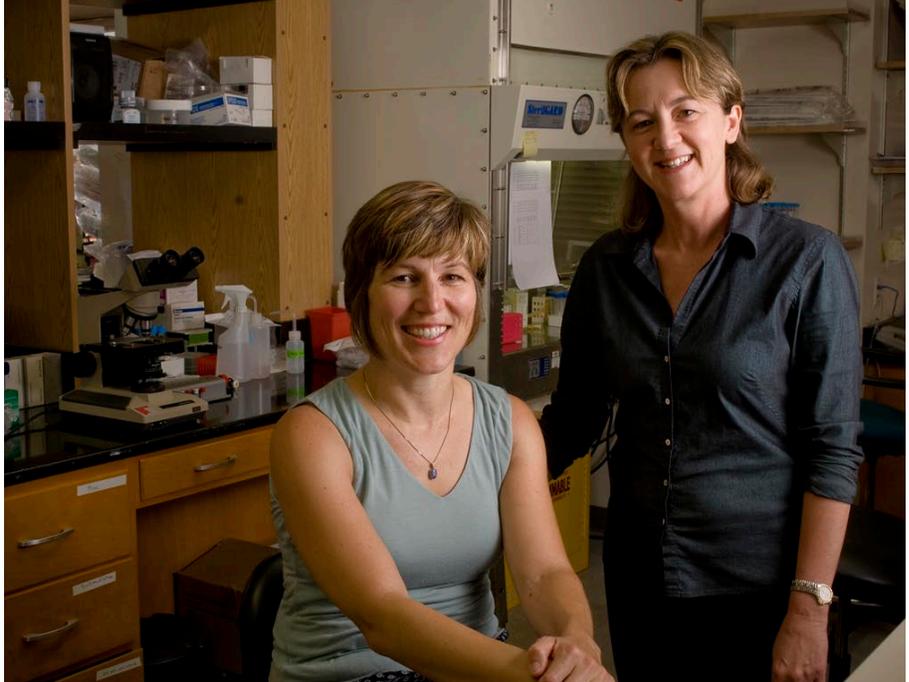
The team's initial laboratory studies indicate that FGF 10 activates the organ's ordinarily dormant progenitor cells—relatively immature cells that promote cell proliferation crucial to tissue repair after surgery or injury. “Could this re-deployment be manipulated therapeutically?” asks Dr. Bellusci. “We hope so.”

“This suggests potentially exciting future clinical applications,” adds Dr. Wang. For example, for children with hemophilia, it

might be possible to import healthy versions of the defective gene that causes their disorder, then stimulate the growth of new live tissue with FGF 10. Or in liver cancer, when so much healthy and diseased organ has to be removed that young lives sometimes are jeopardized, short-term treatment with a synthetic FGF 10 agent might possibly encourage better, faster regeneration of normal liver tissue.

“Let me put it this way,” says Dr. Bellusci, “if I were going to have liver surgery sometime in the future, I might get an injection of FGF 10 beforehand.” He envisions this kind of application being developed over time for children with liver disease.

Elizabeth R. Lawlor, MD, PhD, left,  
and Gay M. Crooks, MD



*Left to right: migrating neural crest cells from a differentiating human embryonic stem cell (hESC) colony; hESC-derived peripheral nerves; and undifferentiated hESC colonies.*

## Gene, Immune and Stem Cell Therapy Program

Director: **Donald B. Kohn, MD\***  
[www.childrenshospitala.org/GISCT](http://www.childrenshospitala.org/GISCT)

The Saban Research Institute's expertise in harnessing the potential disease-fighting power of stem cells was acknowledged in 2007 with three separate grants from the California Institute for Regenerative Medicine (CIRM). Together with its \$2.35 million grant in 2005 for training, Childrens Hospital Los Angeles joins only nine other institutions in California to earn stem cell grants from all CIRM categories. Its four CIRM grants total \$8,427,973.

"This success reflects CIRM's recognition of our existing core strength in human embryonic stem cells (hESC) and our long-term commitment to stem cell research," says Gay M. Crooks, MD\*, director of

the Childrens Hospital Los Angeles Stem Cell Project.

Childrens Hospital is the only stand-alone hospital and the only pediatric hospital to receive a Shared Research Laboratory grant. With the \$2,849,866 grant, it will remodel and equip a dedicated, four-suite laboratory in The Saban Research Institute for hESC research beyond the confines of restricted federal funding. The estimated completion date is July 2008.

Elizabeth R. Lawlor, MD, PhD\*, received a two-year, \$675,001 Scientific Excellence through Exploration and Development (SEED) grant to examine the origins of Ewing's sarcoma, an aggressive tumor that primarily affects children and young adults. Dr. Lawlor, a member of the Division of

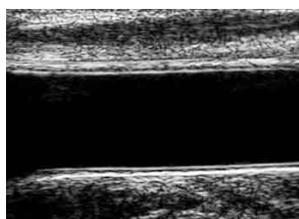
Hematology/Oncology, is a research scientist in the Cancer Program and the Gene, Immune and Stem Cell Therapy Program at The Saban Research Institute.

Dr. Crooks received a four-year, \$2,551,088 Comprehensive Research Grant, awarded to experienced scientists with a record of accomplishment in stem cell research. Her team seeks to better understand the pathways along which the blood and immune system are generated from hESC and to develop the means to expand blood-forming stem cells derived from hESC.

"We believe such innovative research should be available to the children of California," says Dr. Crooks, "and, in fact, that children are likely to be uniquely suited to therapies with stem cells."

\* Faculty member, the Keck School of Medicine of the University of Southern California

Philippe S. Friedlich, MD, left,  
and Vicente Gilsanz, MD, PhD



*In the first study of its kind,  
high-resolution ultrasonography  
was used to measure the carotid  
arterial wall in ECMO survivors.*

## Childrens Imaging Research Program

Director: **Vicente Gilsanz, MD, PhD\***  
[www.childrenshospitala.org/research/imaging](http://www.childrenshospitala.org/research/imaging)

Twenty years ago, Childrens Hospital Los Angeles established one of the first centers in Southern California for Extra Corporeal Membrane Oxygenation (ECMO), which provides pulmonary and/or cardiac bypass support for infants and children in life-threatening respiratory or cardiac failure. Today, the hospital maintains the most active ECMO center in California and one of the most active nationwide, with more than 40 cases annually.

At the same time, the first babies who needed this extraordinary method of life support are now growing older—there are nearly 12,000 survivors of Venous-Arterial

(VA) ECMO in the United States alone. In this procedure, the right carotid artery, which supplies oxygen to the developing brain, is tied off and the ECMO technology takes over. The left carotid artery is intact and able to provide sufficient blood flow.

In 2007, Philippe S. Friedlich, MD\*, medical director of the Neonatal and Infant Critical Care Unit in the Center for Fetal and Neonatal Medicine at Childrens Hospital, and Vicente Gilsanz, MD, PhD, launched the first study of the impact of living with one carotid artery after ECMO use. Thirty-one former VA ECMO patients from Childrens Hospital, now 12 to 20 years old, participated. Using high-resolution ultrasonography to measure the carotid arterial wall, the

investigators found a significant increase in the thickening of the wall in the ECMO survivors compared with 31 healthy subjects.

While the difference was not alarming, and most ECMO patients are doing well, Dr. Friedlich says long-term studies are needed to better understand the lifelong impact on cardiovascular health. “As pediatricians, we have an obligation to participate in the understanding of how pediatric diseases affect survivors into their adult years,” he notes. This collaborative study is in keeping with the spirit of ECMO itself, he adds—a technology made possible by the combined talents of neonatologists, pediatric surgeons, radiologists and specially trained nurses.

Kevin A. Nash, PhD



Left to right: a culture of a fluorescent organism closely related to the tuberculosis (TB) bacterium; a culture of the TB vaccine (“BCG”); and a scanning electron micrograph of TB bacteria. Far right image courtesy of Janice Carr, Centers for Disease Control and Prevention.

## Microbial Pathogens Initiative

Director: **Wilbert H. Mason, Jr., MD, MPH\***  
[www.childrenshospitala.org/research/microbial](http://www.childrenshospitala.org/research/microbial)

Kevin A. Nash, PhD\*, has spent the past decade trying to unravel the difficult problem of drug resistance: the ability of some disease-causing microorganisms, such as bacteria, to survive and even multiply in the presence of drugs designed to kill them.

Lately, he’s been tackling the problem from another angle, asking which processes make microorganisms less susceptible to antibiotics. It’s a subtle but important difference, especially when facing down one of the most important single causes of preventable death worldwide—tuberculosis (TB).

Dr. Nash’s laboratory has been active in discovering multiple processes in

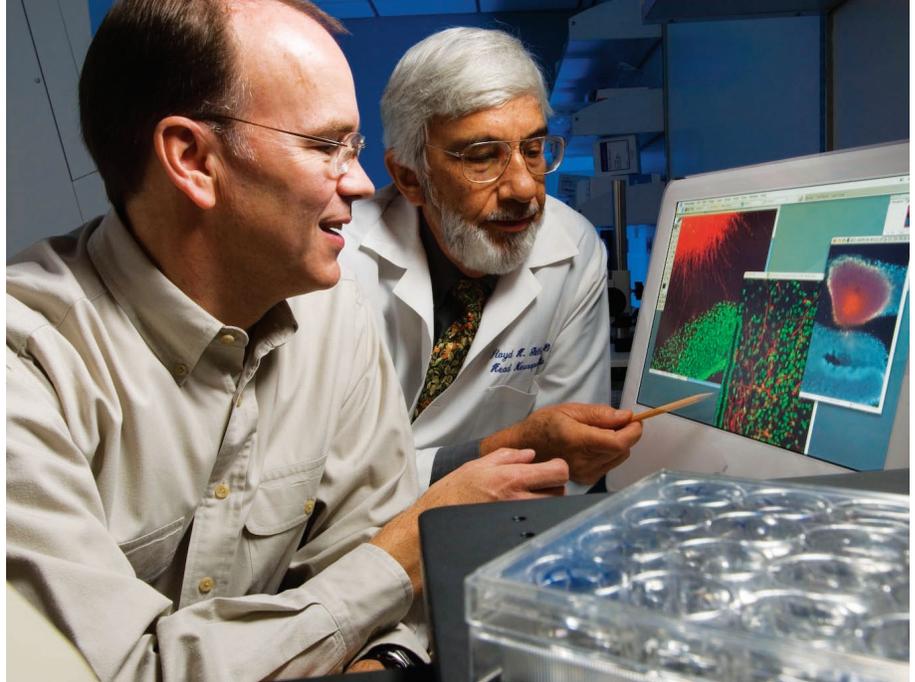
mycobacteria (a large family of hardy bacteria resistant to many commonly used antibiotics), including the causative agent of TB. Recently, the team was instrumental in the characterization of a novel drug resistance gene and now is working to understand how that gene is being regulated. “Then we can begin to develop strategies to get around it,” he says.

Such strategies are crucial because each year there are about eight to nine million new cases of symptomatic TB, which lead to two to three million deaths—more than all forms of cancer combined. Dwarfing these numbers: the estimated two billion people infected with TB bacteria and possibly 50 million with drug-resistant organisms.

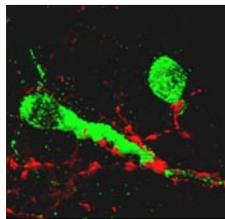
Dr. Nash also is concerned about a collection of diseases caused by rapidly growing mycobacteria (RGM), which are very difficult to treat. He has discovered that some RGM have “cryptic” or hidden drug resistance, which can actually be triggered by chemotherapy.

His investigations are motivated by the fact that TB is preventable. “We do this work in the hope it will make a difference to people with TB and other microorganisms, and will open up new treatment options.” To pursue that goal, he has received funding from the National Institute of Allergy and Infectious Diseases of the National Institutes of Health.

\* Faculty member, the Keck School of Medicine of the University of Southern California



Richard B. Simerly, PhD, left,  
and Floyd H. Gilles, MD



*Brain function depends on the ability of nerve cell axons (red) to form effective synaptic connections with a variety of target cells (green).*

## Neuroscience Program

Director: **Richard B. Simerly, PhD\***  
[www.childrenshospitala.org/research/neuroscience](http://www.childrenshospitala.org/research/neuroscience)

Scientists increasingly agree that behavioral conditions such as autism—a brain disorder that generally appears before age three—have their origins in the development of brain circuits. The Neuroscience Program has placed a top priority on defining the critical signaling pathways that direct the development of these connections to better understand how to diagnose and treat patients when this process goes awry.

“Although we are witnessing dramatic progress in our insights into how brain circuits are formed, it remains a monumental task to translate these novel findings from basic research into therapies,” says Richard B. Simerly, PhD, program director.

To bridge this gap, Childrens Hospital Los Angeles has created the Institute for the Developing Mind (IDM), which will bring together physicians and researchers from across Southern California to generate improved methods of clinical assessment and treatment. “The IDM will help us make the connections between new knowledge and the children who need it,” says Floyd H. Gilles, MD\*, head of the Division of Neurology and director of the Childrens Brain Center.

In addition to fostering collaborations at Childrens Hospital, the University of Southern California and the California Institute of Technology, the IDM will forge links with policy makers and community advocacy groups. Research is a vital part

of the equation going forward. “In addition to strengths in psychology, neuroimaging and neuroscience we currently are building at Childrens Hospital,” says Dr. Simerly, “we need to expand our expertise in clinical neurogenetics and behavioral research.”

This fall, the IDM will launch its first clinical diagnostic and treatment services at Childrens Hospital with the Boone Fetter Clinic for Development and Learning, funded by a \$1 million gift from the Boone Family Foundation. Key supporters of neuroscience research at The Saban Research Institute include The Associates Endowment for Neurosciences and Imaging Research; the USC Provost’s office; and Cheryl Saban, PhD, Haim Saban and the Saban Family Foundation.



# 04

## CLINICAL RESEARCH CENTERS



Clinical research is an essential element in the scientific continuum at Childrens Hospital Los Angeles, with laboratory and clinical researchers routinely feeding into each other's work. Under investigation are new therapies, treatment ideas and technologies that will change the future of health care for children everywhere.

### Center for Endocrinology, Diabetes and Metabolism

Director: **Francine R. Kaufman, MD\***

[www.childrenshospitala.org/research/diabetes](http://www.childrenshospitala.org/research/diabetes)

The seeds of an idea often take root with a single question—*Why?*—such as the day Anna M. Butturini, MD\*, from the Division of Hematology/Oncology at Childrens Hospital Los Angeles, shared her findings with fellow researchers that children who are obese when diagnosed with high-risk leukemia have a greater chance of relapse.

Steven D. Mittelman, MD, PhD\*, a physician-scientist in the Center for Endocrinology, Diabetes and Metabolism, couldn't stop wondering: Was the cause the biological effects of obesity or some aspect of treatment? He joined forces with Nora C. Heisterkamp, PhD\*, whose studies focus on the underlying biology of leukemia.

Working in the laboratory, in 2007 the team found that obesity increases leukemia relapse in mice after chemotherapy. This raises the concern that the way some chemotherapy drugs are dosed to overweight children may lead to decreased efficacy. Next are pharmacological studies to explore chemotherapy levels in fat and the idea that fat cells—once considered inactive—actually participate in disease processes. "This is a very young area of research that we're excited to pursue," says Dr. Mittelman.

In 2007, he received a Research Career Development Award from the Department of Pediatrics, along with funding from the TREC Research Center (Transdisciplinary Research on Energetics and Cancer) at the University of Southern California.

*Above: cross-section of brain tissue.*

*Left: Madison Bosse demonstrates virtual reality at Childrens Hospital Los Angeles. See page 14.*

### Childrens Center for Cancer and Blood Diseases

Director: **Stuart E. Siegel, MD\*** Co-director: **Donald B. Kohn, MD\***

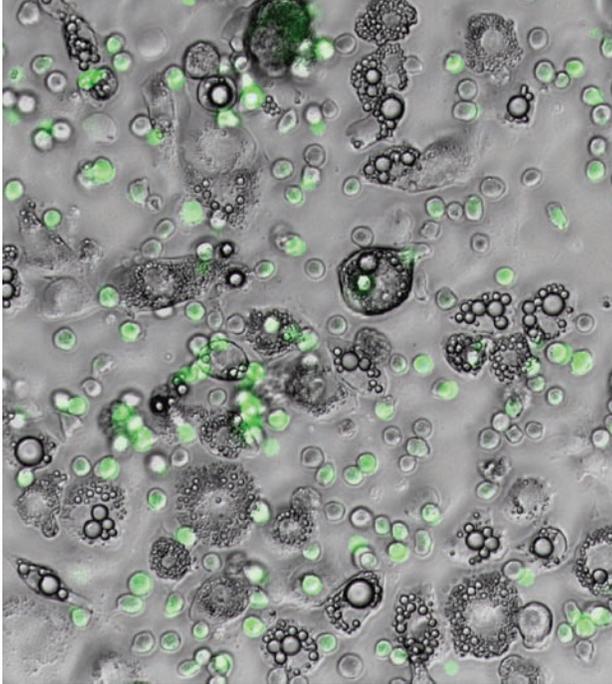
[www.childrenshospitala.org/research/CCCB](http://www.childrenshospitala.org/research/CCCB)

Jonathan Finlay, MB, ChB\*, may be the world's most determined advocate for avoiding radiation therapy in children with brain cancer, the leading cause of cancer-related death in children under 16 years old. A frequent part of treatment plans worldwide, brain irradiation can have devastating effects on intellectual development. So Dr. Finlay has spent the past 16 years creating alternatives as leader of the "Head Start" Brain Tumor Consortium.

This international group of more than 40 participating centers, based at Childrens Hospital Los Angeles, develops and tests innovative protocols for young children newly diagnosed with malignant brain tumors. The current protocol uses five months of standard, intensive doses of chemotherapy, followed by a single course of extremely high dose drug treatment (equivalent to a year's worth in just six days) and a blood cell transplant with the patient's own previously collected and preserved blood cells. Children under age six who achieve a complete remission receive no irradiation. Those ages six to 10 receive much lower than the standard doses.

"At least 50 to 60 percent of the children stay in remission, without cognitive deficits. That's good, but not good enough," says Dr. Finlay, clinical director of the Neural Tumors Program at Childrens Hospital. Another "Head Start" study will begin in the next two years, adding mild post-transplant drug treatment to target any surviving cancer cells. In collaboration with other North American pediatric cancer programs, Dr. Finlay also will initiate Phase I clinical trials in 2008 to establish maximum-tolerated dosage of potential new drugs. It is one more facet of his strategy: better drugs, utilized in better ways.

\* Faculty member, the Keck School of Medicine of the University of Southern California



*The relationship between obesity and leukemia intrigues Steven D. Mittelman, MD, PhD. Above are leukemia cells (green), growing in culture with fat cells. See page 13.*

### Childrens Clinical Investigations Center

Director: **Edward D. Gomperts, MD\***  
[www.childrenshospitala.org/research/clic](http://www.childrenshospitala.org/research/clic)

Wearing head-mounted display units that resemble futuristic helmets, children race luge-style down snowy mountains. They lean their bodies left and right, skirting trees and boulders obstructing their course. Welcome to the world of Virtual Reality (VR), where children can get so immersed in playful 3-D environments they become oblivious to pain and the associated distress of invasive medical procedures.

Jeffrey I. Gold, PhD\*, recipient of the first Clinical Research Academic Career Development Award from The Saban Research Institute in 2007, is pioneering an exploration of why VR works. Dr. Gold is a member of the Comfort, Pain Management and Palliative Care team in the Department of Anesthesiology Critical Care Medicine at Childrens Hospital Los Angeles.

His study, which began this summer, looks at how VR affects the neuro-activity of pain. Utilizing functional Magnetic Resonance Imaging (fMRI)—which shows specific regions of the brain lighting up as mental processes occur—Dr. Gold administers a thermal pain stimulus at random periods in combination with the presence or absence of VR. To evaluate the pain-reducing components of VR, he repeats the experiment as subjects play VR games. Because the study triggers pain, it only will enroll adults. If proven safe, it will be repeated with children.

“Children who undergo invasive medical procedures report as much distress as patients with generalized anxiety disorder,” says Dr. Gold. If his research shows that gaming not only reduces activity in brain regions linked to pain perception, but also stimulates those associated with an analgesic effect, the results could make a strong argument for the therapeutic use of VR.

### The Heart Institute

Director: **Vaughn A. Starnes, MD\***  
[www.childrenshospitala.org/heart](http://www.childrenshospitala.org/heart)

In the first-ever randomized surgical trial for pediatric heart disease, cardiac surgeons and cardiologists are asking how to ensure the optimal survival of babies born with hypoplastic left heart syndrome (HLHS). “This kind of study takes a tremendous commitment. It’s the only way to truly answer such a complex question,” says Alan B. Lewis, MD\*, co-principal investigator at Childrens Hospital Los Angeles with Vaughn A. Starnes, MD.

The Single Ventricle Reconstruction (SVR) Study, funded by the National Heart, Lung and Blood Institute of the National Institutes of Health, involves Childrens Hospital and 14 other members of the Pediatric Heart Network, a consortium of major pediatric cardiac institutions.

In HLHS, which is fatal without corrective surgery, the left ventricle is missing or underdeveloped. The study’s goal: determine which of two shunt positions, if either, offers advantages for patients undergoing the Norwood Procedure, the first stage in repairing a single ventricle heart. It involves enlarging the aorta, opening the wall between upper chambers of the heart and inserting a shunt or small connector to deliver blood to the lungs.

Each child in the study is randomly assigned a shunt position. The surgeon can, however, override that decision. Participants will be followed for 14 months, with plans for long-term follow-up, given funding. Research Coordinator Nancy Pike, RN, PNP, PhD, is working to enroll 35 families at Childrens Hospital by this fall out of a total of 466 nationwide.

### Childrens Orthopaedic Center

Director: **Vernon T. Tolo, MD\***  
[www.childrenshospitala.org/research/ortho](http://www.childrenshospitala.org/research/ortho)

Inside the John C. Wilson, Jr., Motion Analysis Laboratory at Childrens Hospital Los Angeles, cameras are braced on every wall and sensor blocks dot the floor. As youngsters walk about, computers like those used for film animation recreate their gaits in moving three-dimensional images.

By seeing a full range of joint and muscle activity occur simultaneously, surgeons can identify multi-level problems and design one, all-encompassing surgery. Studies have shown that fully 89 percent of preoperative plans change when surgeons utilize motion analysis. “This diagnostic tool is invaluable for patients who otherwise would have multiple operations to correct one problem at a time,” says Tishya Wren, PhD\*, director of research for the Motion Analysis Laboratory.

Yet even the best insurance policies often exclude motion analysis as a reimbursable test. Now Dr. Wren is in the fourth year of a five-year randomized study designed to document that motion analysis can improve surgical outcomes.

\* Faculty member, the Keck School of Medicine of the University of Southern California



*Far left: Early visual development is the subject of research in The Vision Center. Left: Special muscle sensors attached to Rory Pankuk's legs feed data to a computer in the John C. Wilson, Jr., Motion Analysis Laboratory.*

Working with doctors at Shriners Hospitals for Children—Los Angeles who want access to the insights from motion analysis, Dr. Wren provides a gait analysis report on half of their patients who have cerebral palsy, which is characterized by poor muscle control and movement abnormalities. If her study proves that the sophisticated analysis leads to better outcomes, then insurance providers may be more inclined to reimburse for it—and patients can gain from more appropriate, better surgeries.

### The Vision Center

Director: **Mark S. Borchert, MD\***  
[www.childrenshospitala.org/vision](http://www.childrenshospitala.org/vision)

Kristina Tarczy-Hornoch, MD, DPhil\*, is interested in how the human brain learns to see—and what can go wrong during development. One of her investigations concentrates on strabismus or eye misalignment, which often results from farsightedness and frequently leads to serious vision deficit. When adults develop it, their brains permanently produce double vision. “But the very young brain ingeniously adapts, learning to suppress images that enter through one eye or the other,” explains Dr. Tarczy-Hornoch, director of the Vision Development Institute, one of six institutes in The Vision Center at Childrens Hospital Los Angeles.

In collaboration with Vincent Chen, PhD\*, from the Department of Radiology, she is using the latest in neuro-imaging technology—functional Magnetic Resonance Imaging (fMRI)—to pinpoint where visual activities occur in the brains of children, adolescents and adults who have signs of visual suppression. The investigators first expose the study subjects to visual stimuli that initiate suppression, then eliminate one of those stimuli. The fMRI images reveal if and where the visual process changes.

Investigations like this may lead to therapies for youngsters whose eyes and brain aren’t in sync.

“A child’s brain is so potentially modifiable,” says Dr. Tarczy-Hornoch, “that we believe there must be ways to make this adaptability work for the child’s vision instead of against it.”

### USC-CHLA Institute for Pediatric Clinical Research

Principal Investigators: **Roberta G. Williams, MD\***, and **Stuart E. Siegel, MD\***  
[www.ipcr.us](http://www.ipcr.us)

The road from initial hope to clinical application can be long for new anti-cancer drugs. Nevertheless, Min H. Kang, PharmD\*, director of the Leukemia Preclinical Testing Lab in the USC-CHLA Institute for Pediatric Clinical Research (IPCR), is excited about the potential for one new drug to benefit children with leukemia. Her research is building evidence that ABT-737, a small molecule developed by Abbott Laboratories, has promise in the arsenal against acute lymphoblastic leukemia (ALL).

Dr. Kang has shown that this exciting new drug significantly enhances the power of a combination of chemotherapeutic drugs commonly used against ALL. By inhibiting the over-expression of proteins that protect the cancer from cell death, ABT-737 essentially destroys leukemia’s armor and exposes more of it to frontal attack.

Today, 80 percent of children with ALL are cured. Still, one child in five with ALL will die of the disease. “ABT-737 may be part of the answer,” says Dr. Kang. “In laboratory studies, it shows dramatic activity. It is the most exciting new agent I have researched so far.”

In 2007, her findings were published in *Blood*, the journal of the American Society of Hematology, in an article authored with C. Patrick Reynolds, MD, PhD\*, director of the IPCR’s Developmental Therapeutics Program. Dr Kang’s paper paves the way for clinical trials of this new approach to cancer chemotherapy to be developed for children with ALL.

# RESEARCH EDUCATION

*The Class of 2007 of the High School Research Education Program: Diamond LaBon, Christopher Ndubuizu, Paul Zapata, Oscar Calzada, Colleen Gonzales, Betsy Diaz, Deborah Ortiz, Lauren Kay, Ana Lopez, Andres Artiga and Karen Amaya.*



## High School & Undergraduate Education Programs

The Saban Research Institute is addressing a nationwide shortfall of minorities in biomedical sciences by expanding the Class of 2007 in its High School Summer Research Education Program and by launching a new nationwide research training program for undergraduates—both the vision of Emil Bogenmann, PhD\*, head of research education.

The Undergraduate Short Term Education Program for Underrepresented Persons (STEP-UP) received a five-year, \$970,000 grant from The National Institute of Diabetes and Digestive and Kidney Diseases of the National Institutes of Health. Dr. Bogenmann served as principal investigator of the four-center pilot program. Sixty-five students concluded their 10-week internship in August, having been exposed to research in diabetes, endocrinology, metabolism and obesity, among other scientific areas.

At the same time, 11 students from Los Angeles-area public high schools took part in the second High School Research Education Program, up from eight students a year ago. During the six-week

summer internship, students assisted in investigations at The Saban Research Institute that focused on lung development and repair, gene therapy, heart regeneration, the blood-brain barrier, bone marrow transplants, infectious diseases, HIV transmission and the biology of arenaviruses.

This year, each student was assigned a college mentor in addition to a scientific mentor. The interns attended weekly college prep meetings and research roundtables. Primary funding for the first two years has been provided by Lori and Ted Samuels, chair of The Saban Research Institute Committee and a member of the Childrens Hospital Los Angeles Board of Trustees. Other funding came from The Leonetti/O'Connell Family Foundation and the Kayne Foundation.

Together, the two education programs represent a unique opportunity. "Our high school students can continue to gain laboratory experience while in college by becoming STEP-UP students," says Dr. Bogenmann. "We're creating a pipeline for future scientists."

\* Faculty member, the Keck School of Medicine of the University of Southern California

# AWARDS AND NEWS



## 2007 Intramural Research Awards

Each year, The Saban Research Institute and the Department of Pediatrics at Childrens Hospital Los Angeles provide these competitive awards to encourage basic and clinical research by faculty. This is where new ideas are born.

### Post-doctoral Award

#### **Tanja Gruber, MD, PhD**

Division of Hematology/Oncology  
Mentor: Markus Müschen, MD, PhD\*

“Activation Induced Cytidine Deaminase as a Causative Agent of Therapy Resistance in Philadelphia Chromosome Positive Acute Lymphoblastic Leukemia (ALL)”

### Academic Career Development Award

#### **Susan Wu, MD**

Division of General Pediatrics  
Mentor: Wilbert H. Mason, Jr., MD, MPH\*

“Nebulized Hypertonic Saline for Treatment of Viral Bronchiolitis”

#### **Elisabeth Raab, MD\***

Division of Neonatology  
Mentor: Henri R. Ford, MD\*

“Impact of Probiotics on Feeding Tolerance and Incidence of Bacteremia in Neonates With Gastrostomies”

### Career Development Award

#### **Sebastien Bouret, PhD\***

Neuroscience Program  
Mentor: Richard B. Simerly, PhD\*

“Prenatal Nutrition and Hormone-Dependent Neurogenesis in Hypothalamus”

#### **Yong-mi Kim, MD\***

Division of Hematology/Oncology  
Mentor: Markus Müschen, MD, PhD\*

“Identification and Functional Analysis of ALL Stem Cells”

### Clinical Research Academic Career Development Award

#### **Jeffrey I. Gold, PhD\***

Departments of Anesthesiology Critical Care Medicine and Pediatrics

Mentors: Ernest Katz, PhD\*;  
Marvin D. Nelson, Jr., MD\*

“Using fMRI to Examine the Neurobiological Basis of Virtual Reality Pain Attenuation”

### Graduate Student Award

#### **Stefano Da Sacco**

Developmental Biology  
Mentors: Roger De Filippo, MD\*;  
David Warburton, DSc, MD, FRCP\*

“Isolation of Kidney Progenitor Cells from Human Amniotic Fluid and Evaluation of Their *In Vitro* Regenerative Potential”

### Career Development Fellowship Award

#### **Vanessa Taupin, PhD**

Gene, Immune and Stem Cell Therapy  
Mentor: Paula Cannon, PhD\*

“CCRS Co-Receptor Knock Out in Human HSC as an Anti-HIV Gene Therapy”

#### **Christine Burt-Solarzano, MD**

Neuroscience Program  
Mentors: Mitchell E. Geffner, MD\*;  
Richard B. Simerly, PhD\*

“Perinatal Nutrition and the Maturation of Glucose Metabolism and Hypothalamic Feeding Circuits in Mice”

#### **Saro Armenian, MD**

Division of Hematology/Oncology  
Mentors: Richard Sposto, PhD\*;  
Smita Bhatia, MD (City of Hope)

“Late Cardiac Complications in Hematopoietic Stem Cell Transplant Recipients”

#### **Brynie Collins, MD**

Division of Gastroenterology  
Mentors: Edward D. Gomperts, MD\*;  
Henry Lin, MD

“Defining the Prevalence and Role of Small Intestinal Bacterial Overgrowth in Children with Chronic Abdominal Pain”

## International Collaboration Begins

Childrens Hospital Los Angeles launched a partnership in 2007 with The Saban Children's Hospital in Be'er Sheva, Israel, to share interdisciplinary research and patient care methods with the goal of improving children's health care worldwide.

Initial funding for the innovative collaboration has come from Cheryl Saban, PhD, Haim Saban and the Saban Family Foundation, whose generous gift in 2003 funded the construction of the 88,500-square foot Saban Research building at Childrens Hospital Los Angeles.

The Los Angeles-Be'er Sheva Saban Collaborative Program began this year with an exchange of pediatric residents. Exchanges of faculty, research fellows and other health-care professionals are expected to start in 2008.

“By connecting talented physicians in Israel with one of the most active, productive pediatric research facilities in the United States,” says Dr. Saban, a member of the Childrens Hospital Los Angeles Board of Trustees, “we are taking advantage of a unique opportunity to tap into the strengths of each community to expand global pediatric research.”

# HONOR ROLL AND ENDOWMENTS

## Honor Roll of Donors from July 1, 2006 to June 30, 2007

We are proud to recognize the following donors who made gifts of \$1,000 and above during the last fiscal year to advance research at Childrens Hospital Los Angeles. These philanthropic partners provided critical support that, coupled with federal sources such as the National Institutes of Health, helped to further key studies in established programs and foster new ideas in expanding areas such as clinical research. The Saban Research Institute of Childrens Hospital Los Angeles and its dedicated investigators are grateful to these individuals, foundations and businesses for sharing our dream of a world without pediatric disease. For more information on how you can make a difference, please contact Melany Duval, associate vice president of Major and Planned Gifts, at 323-361-1705 or mduval@chla.usc.edu.

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