

Feb. 21, 2014

The Saban Research Institute Annual Symposium

REGENERATIVE MEDICINE AND CELLULAR THERAPIES:

Inventing the Future for Children



DIRECTOR'S WELCOME

D. Brent Polk, MD

The Saban Research Institute of Children's Hospital Los Angeles is dedicated to understanding the developmental origins of health and disease; one path that holds great promise in this regard is regenerative medicine and cellular therapies. As technology enables innovative imaging capabilities and more flexible data management, processing and analyses, scientists are empowered to uncover how the body builds and renews itself so that we may harness this knowledge to create better health.

Recognizing our role in this vital field, Children's Hospital has partnered over the years with the University of Southern California and our many supporters to build strength and influence through the Developmental Biology and Regenerative Medicine Program. Most notably, since 2003 the Pasadena Guild of Children's Hospital Los Angeles has been a generous supporter of this program. Today, I am honored to announce the Pasadena Guild Endowed Chair in Developmental Biology and Regenerative Medicine, the inaugural holder of which will be David Warburton, OBE, DSc, MD, MMM, FRCP, FRCS, FRCPC. Warburton has led The Saban Research Institute's Developmental Biology and Regenerative Medicine Program since its inception, growing the program to include more than 25 collaborating scientists ranging from talented junior faculty to established and internationally recognized experts with publications in high-impact journals such as Nature, Nature Cell Biology, Development and the New England Journal of Medicine.

The many accomplishments of the Developmental Biology and Regenerative Medicine Program have brought visibility to Children's Hospital and The Saban Research Institute, contributing to our outstanding reputation as a leading pediatric academic medical center and bringing key sources of federal and philanthropic funding to further support our mission of creating hope and building healthier futures.

We are now poised to expand our research enterprise through the Regenerative Medicine and Cellular Therapies focus area, one of three synergistic areas identified

by Children's Hospital leadership for strategic investment and growth to accelerate our discovery, development and delivery of improved clinical outcomes.

Our alignment with USC in this important field will accelerate expansion of expertise and research capabilities critical to leveraging our current strengths while branching into new and diverse fields of study. Most notably, collaboration on key initiatives such as USC Stem Cell and the Children's Hospital Los Angeles–University of Southern California Translational Biomedical Imaging Laboratory, with Andrew McMahon, PhD, provost professor and director of the Eli and Edythe Broad Center for Regenerative Medicine and Stem Cell Research at the Keck School of Medicine of USC, and Scott Fraser, PhD, USC provost professor of Biological Sciences and Biomedical Engineering and director of Science Initiatives, are leading to increased opportunities for truly innovative multidisciplinary research and transformative recruitments.

I am also pleased to extend my sincerest gratitude to the many philanthropists with us today and to those who could not attend. Your commitment to the health and well-being of children is a vital part of our many successes and our future goals.

As we gather today to hear from an outstanding lineup of nationally recognized experts on regenerative medicine and cellular therapies, I invite everyone to envision a revolutionized health care system driven by discoveries in Regenerative Medicine and Cellular Therapies.

D. Brent Polk, MD
Chair of the Department of Pediatrics, physician in chief and vice president for Academic Affairs, Children's Hospital Los Angeles; director, The Saban Research Institute of Children's Hospital Los Angeles; professor and chairman of Pediatrics, vice dean for Child Health and professor of Biochemistry and Molecular Biology, the Keck School of Medicine of the University of Southern California (USC)



David Warburton, OBE, DSc, MD, MMM, FRCP, FRCS, FRCPC

Symposium Co-organizer

David Warburton, OBE, DSc, MD, MMM, FRCP, FRCS, FRCPC, is a global thought leader in child health, regenerative medicine and cellular therapeutics. He currently leads the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute of Children's Hospital Los Angeles. His personal research program focuses on the lung as the rate-limiting step for adaptation of human infants to breathing air. His work and the work of his scientific colleagues at The Saban Research Institute is based on the overarching insight that we all come from one cell, and as the information encoded within the genome unfolds we develop into a multicellular, self-assembling, self-repairing biological machine called a human being. He therefore avers that developmental studies in children hold the key to inventing future cures for all major diseases that afflict humans over a life span. Warburton holds a medical degree as well as a higher doctorate of science from the University of London, is an elected member of numerous academies and royal colleges and has been created an Officer of the Order of the British Empire by Her Majesty, Queen Elizabeth II. He has served on the medical staff of Children's Hospital Los Angeles and the faculty of the University of Southern California (USC) for 35 years.



Robert Seeger, MD, MS

Symposium Co-organizer

After graduating with a master's degree and a medical degree from the University of Oregon Medical School in 1966, Robert Seeger, MD, MS, became a pediatric intern and resident at the University of Minnesota Medical School and Hospitals (1966-1968). Seeger then focused on laboratory research in immunology at the National Institutes of Health (1968-1971) and in cancer immunology at University College London (1972-1973). In 1974, Seeger joined the Department of Pediatrics at the University of California, Los Angeles (UCLA) School of Medicine, where he became a professor of Pediatrics and associate director for Clinical Research of the UCLA Jonsson Comprehensive Cancer Center. His research focused on neuroblastoma, a childhood cancer, and while working with molecular biologists (1984-1985) Seeger made the seminal discovery that the MYCN oncogene in neuroblastoma tumors could be used to predict survival. This was the first demonstration, for any human tumor, that a cancer gene could predict outcomes. In 1989, Seeger became professor of Pediatrics in the Division of Hematology-Oncology at the Keck School of Medicine of USC, and head of the Cancer Research Program at Children's Hospital Los Angeles. Seeger and colleagues developed new strategies for treating children with high-risk neuroblastoma, which was shown in a nationwide study to improve survival and continues to be the worldwide "gold standard" of treatment. His laboratory's research was recognized in 2000 with funding of a Program Project Grant (now in its 14th year) from the National Cancer Institute. Seeger has published 170 peer-reviewed papers throughout his career. He received the H. Russell Smith Award for Innovation in Pediatric Biomedical Research (2001) and was president of the international Advances in Neuroblastoma Research meeting (2006). In 2011, Seeger received the USC Mellon Mentoring Award, presented to outstanding faculty mentors.

THE SABAN RESEARCH INSTITUTE OF CHILDREN’S HOSPITAL LOS ANGELES: Research Strategy

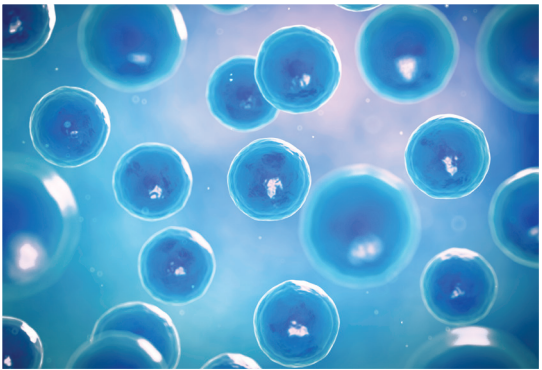
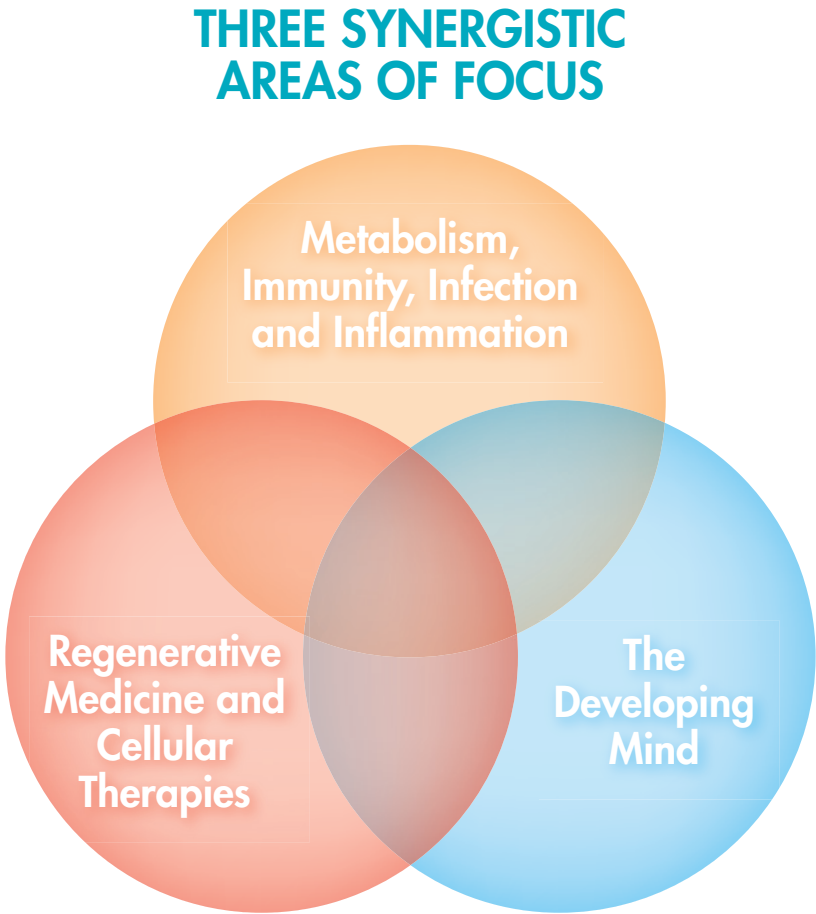
Unifying Research Theme Developmental Origins of Health and Disease

The Saban Research Institute integrates basic, clinical and translational research around the Developmental Origins of Health and Disease. We now know that all people are the result of a complex interaction between their own genetics and the environment in which they live. We also realize that adult diseases have their roots in childhood, offering us a unique opportunity for impact. This principle unifies our diverse research portfolio and drives the central mission of pediatric medicine and scientific innovation at Children’s Hospital Los Angeles.

Three Synergistic Areas of Focus

- The Institute for the Developing Mind
- Regenerative Medicine and Cellular Therapies
- Metabolism, Immunity, Infection and Inflammation

The unifying theme of the Developmental Origins of Health and Disease supports three synergistic areas of focus designed to address pressing national child health issues and further align our goals. These areas are also the basis for expanding our common language, which is required for accelerated progress and problem solving. These three areas support targeted growth in, and foster increased interaction among, our existing scientific programs and priorities. We are thereby building a critical mass of investigators with unlimited opportunities for integration and partnership.



REGENERATIVE MEDICINE AND CELLULAR THERAPIES

Recent advances in regenerative medicine and cellular therapies hold great promise in many areas for improving child and adult health; organ regeneration, stem cell-based therapy and tissue engineering could be lifesaving. The field of cellular therapeutics and tissue engineering is expanding rapidly, promising an exciting array of potential applications. Researchers and clinicians are developing treatments for many genetic conditions as well as diseased, mechanically injured or metabolically deficient tissues, and Children’s Hospital Los Angeles is poised to become a global leader in the field of regenerative medicine and cellular therapies.

Research discoveries already being generated at Children’s Hospital Los Angeles as well as those discoveries anticipated by investigators in the Regenerative Medicine and Cellular Therapies focus area will provide transformative opportunities to significantly impact unmet needs through advancements in basic stem cell biology, applied stem cell therapies and tissue engineering.

At Children’s Hospital Los Angeles, advances have already led to exciting innovations garnering national attention, such as achievements in growing tissue-engineered human large intestine to help babies who are born with congenital problems of the intestine or have part of the intestine removed soon after birth due to complications of prematurity; work in kidney regeneration demonstrating that amniotic fluid stem cells can slow progression of kidney disease; and lung regeneration technologies. These advances support our goal to be the first and best in research leading to new treatments for improved outcomes. The mission for Regenerative Medicine and Cellular Therapies at Children’s Hospital is

to discover therapeutic targets for a wide array of diseases. These discoveries will be not only scientifically significant but also eminently translatable to adult diseases, and will have a worldwide and multigenerational impact.

In order to build upon the very significant scientific and clinical breakthroughs that have already been achieved at Children’s Hospital, and to optimize and speed translation of our basic discoveries into the clinic, we are developing strengths in Regenerative Medicine and Cellular Therapies through three initial priority areas: stem cell biology, stem and cellular therapeutics, and tissue engineering.

Today’s symposium, “Regenerative Medicine and Cellular Therapies: Inventing the Future for Children,” is an opportunity to learn more about this exciting and important area of focus as we move forward, achieving our best together.

SCHEDULE

Session I:
Scientific Basis for Regenerative Medicine

9 – 9:30 a.m.
Continental Breakfast and Check-in
The Saban Research Building lobby

9:30 – 9:40 a.m.
Symposium Welcome, Session I Moderator
D. Brent Polk, MD, AGAF, The Saban Research Institute of Children’s Hospital Los Angeles; Keck School of Medicine of the University of Southern California (USC)

9:40 – 10 a.m.
From One Cell to a Self-Assembling, Self-Repairing Human Being: How Do We Invent Medical and Surgical Cures Out of That Life-Giving Process?
Keynote Speaker: David Warburton, OBE, DSc, MD, MMM, FRCP, FRCS, FRCPCH, The Saban Research Institute of Children’s Hospital Los Angeles; Keck School of Medicine of USC

10 – 10:30 a.m.
Generation and Regeneration of the Cardiopulmonary System
Keynote Speaker: Ed Morrisey, PhD, Penn Institute for Regenerative Medicine, Children’s Hospital of Philadelphia and University of Pennsylvania

10:30 – 10:45 a.m.
Tissue Engineering Human Intestine: Function and Future
Tracy Grikscheit, MD, The Saban Research Institute of Children’s Hospital Los Angeles; Keck School of Medicine of USC

10:45 – 11 a.m.
Heart Regeneration After Injury: Fishing for Answers
Ellen Lien, PhD, The Saban Research Institute of Children’s Hospital Los Angeles; Keck School of Medicine of USC

11 – 11:15 a.m.
Break
Light refreshments

11:15 – 11:30 a.m.
Developmental Impact on Adult Lung Diseases
Wei Shi, MD, PhD, The Saban Research Institute of Children’s Hospital Los Angeles; Keck School of Medicine of USC

11:30 – 11:45 a.m.
Lung Repair and Regeneration: Wear and Tear as Life Goes On
Barbara Driscoll, PhD, The Saban Research Institute of Children’s Hospital Los Angeles; Keck School of Medicine of USC

11:45 a.m. – 12:05 p.m.
Luncheon Remarks
Jon Thomas, PhD, JD, chairman, Independent Citizens’ Oversight Committee for the California Institute for Regenerative Medicine

12:05 – 12:30 p.m.
Presentation of the Pasadena Guild Endowed Chair in Developmental Biology and Regenerative Medicine to David Warburton, OBE, DSc, MD, MMM, FRCP, FRCS, FRCPCH

12:30 – 1:30 p.m.
Lunch Served (RSVP required)
Anita S. Watson Courtyard of The Saban Research Building

Sessions are held in the auditorium of The Saban Research Building

Session II:
New Ways to Image Generation and Regeneration in Living Tissues

1:30 – 1:35 p.m.
Moderator
Marvin Nelson, MD, MBA, FACR, Children’s Hospital Los Angeles; Keck School of Medicine of USC

1:35 – 2:05 p.m.
Convergent Strategies for Regenerative Medicine and Cellular Therapies in Living Tissue
Keynote Speaker: Scott E. Fraser, PhD, The Saban Research Institute of Children’s Hospital Los Angeles; Keck School of Medicine of USC

Session III:
Novel Regenerative Solutions: Tissue Engineering and Cellular Therapeutics

2:05 – 2:10 p.m.
Moderator
Henri Ford, MD, MHA, Children’s Hospital Los Angeles; Keck School of Medicine of USC

2:10 – 2:40 p.m.
The Kidney: Generative and Regenerative Solutions for Preventing Chronic Kidney Failure
Keynote Speaker: Andrew McMahon, PhD, Keck School of Medicine of USC

2:40 – 2:55 p.m.
Novel Cell-Based Therapeutic Approaches for Kidney Disease
Laura Perin, PhD, The Saban Research Institute of Children’s Hospital Los Angeles; Keck School of Medicine of USC

2:55 – 3:10 p.m.
Break
Light refreshments

3:10 – 3:40 p.m.
The Brain and Neural Tube: Achieving Closure
Keynote Speaker: Lee Niswander, PhD, University of Colorado

3:40 – 4 p.m.
Using Targeted Nucleases to Precisely Engineer Hematopoietic Stem Cells for Acquired and Genetic Diseases
Paula Cannon, PhD, Keck School of Medicine of USC

4 – 4:30 p.m.
Neural Stem Cells: A Trojan Horse Strategy for Brain Cancers
Karen S. Aboody, MD, City of Hope

4:30 – 4:45 p.m.
Closing Remarks
David Warburton, OBE, DSc, MD, MMM, FRCP, FRCS, FRCPCH
Robert Seeger, MD, MS

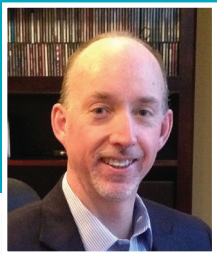
4:45 – 6 p.m.
Reception (RSVP requested)
Anita S. Watson Courtyard, The Saban Research Building

Sessions are held in the auditorium of The Saban Research Building



D. Brent Polk, MD, AGAF

D. Brent Polk, MD, AGAF, is the chair of the Department of Pediatrics, physician in chief and vice president for Academic Affairs, Children's Hospital Los Angeles; director, The Saban Research Institute of Children's Hospital Los Angeles; and professor and chairman of Pediatrics, vice dean for Child Health and professor of Biochemistry and Molecular Biology at the Keck School of Medicine of the University of Southern California (USC). He previously served as chief of Pediatric Gastroenterology, Hepatology and Nutrition, as well as director of the Digestive Disease Research Center at Vanderbilt University in Nashville, Tenn. His research is focused on the regulation of growth and development of the intestines, making important contributions to our understanding of the relationship between inflammation and tumorigenesis. Polk is a member of several professional organizations, including the American Gastroenterological Society, the American Pediatric Society, the American Physiological Society, the American Society for the Biochemistry and Molecular Biology and the Society for Pediatric Research. He is certified by the American Board of Pediatrics and the Subspecialty Boards in Gastroenterology.



Edward E. Morrisey, PhD
Keynote Speaker

Edward E. Morrisey, PhD, is the scientific director of the Penn Institute for Regenerative Medicine and a professor of Medicine and Cell and Developmental Biology at the University of Pennsylvania. Morrisey is an international expert on pulmonary and cardiovascular development and regeneration. The focus of his lab is on defining the mechanisms underlying lung and cardiac development and how these can be harnessed to improve repair and regeneration in these tissues. The ultimate goal is to better understand how signaling and epigenetic pathways regulate cardiopulmonary development and regeneration, which may lead to the development of novel therapeutic approaches for lung and cardiac diseases. He is a member of several National Institutes of Health research consortia related to stem cell and regenerative biology, including the Progenitor Cell Biology Consortium, The Lung Repair and Regeneration Consortium (of which he is the steering committee chair) and the Next-Gen iPSC Consortium. He is also a past associate editor for the Journal of Clinical Investigation.



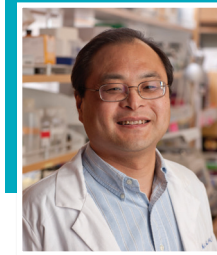
Tracy Grikscheit, MD

Tracy Grikscheit, MD, is a faculty member of the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute of Children's Hospital Los Angeles, an attending surgeon at Children's Hospital Los Angeles and an assistant professor of Surgery at the Keck School of Medicine of USC. She completed her training in general surgery at Massachusetts General Hospital in Boston. During that time she worked with Joseph P. Vacanti, MD, in the Laboratory for Tissue Engineering and Organ Fabrication. Her research, centering on engineered solutions for congenital and acquired intestinal deficits, has been reported in numerous peer-review journals and national and international presentations. Grikscheit completed her surgical training with a pediatric surgery fellowship at Seattle Children's Hospital and Regional Medical Center before joining Children's Hospital Los Angeles. Grikscheit is a funded primary investigator at The Saban Research Institute, continuing her work in tissue engineering and the role of organ-specific stem cells in intestinal development. In addition, she is the surgical director of the intestinal failure team at Children's Hospital and consults with the hospital's Institute for Maternal-Fetal Health.



Ching-Ling (Ellen) Lien, PhD

Ching-Ling (Ellen) Lien, PhD, is a faculty member of the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute of Children's Hospital Los Angeles and assistant professor of Surgery at the Keck School of Medicine of USC. Lien received her bachelor's degree and master's degree in zoology from National Taiwan University, and her doctoral degree in genetics and developmental biology from the University of Texas Southwestern Medical Center at Dallas, training with Eric Olson, PhD. She completed her postdoctoral fellowship with Mark Keating, MD, at Harvard Medical School and Children's Hospital of Boston. Lien's lab studies the molecular mechanisms of heart regeneration in adult zebrafish. She and her colleagues have discovered important growth factors and signaling pathways that regulate cardiomyocyte proliferation during myocardial regeneration and coronary vessel development and regeneration. By comparing the regenerative capacity of zebrafish and mouse hearts, they have attempted to understand why mammalian hearts cannot regenerate. Lien has received career development awards from The Saban Research Institute, the Wright Foundation and the American Heart Association. Her current research is funded by the National Heart, Lung and Blood Institute of the National Institutes of Health (NIH).



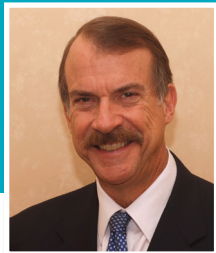
Wei Shi, MD, PhD

Wei Shi, MD, PhD, is a faculty member of the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute of Children's Hospital Los Angeles and a tenured associate professor of Surgery at the Keck School of Medicine of USC. He obtained his medical degree at Zhejiang University, China, and his doctoral degree at University of Tennessee, Memphis. Shi had his postdoctoral training in lung biology at Children's Hospital Los Angeles before he was promoted in 2000 to the rank of assistant professor, and then again to tenured associate professor. He has been studying growth factor signaling in lung development, injury repair and regeneration with the support of the National Institutes of Health and other funding agencies for more than a decade. One of his active projects focuses on developmental antecedents of adult chronic lung diseases.



Barbara Driscoll, PhD

Barbara Driscoll, PhD, is a faculty member of the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute of Children's Hospital Los Angeles and associate professor of Surgery at the Keck School of Medicine of USC. Undergraduate studies at the University of San Diego and doctoral studies at the University of Arizona developed her interest in the molecular mechanisms that underlie disease. She came to Children's Hospital and USC as a postdoctoral fellow, where she continued investigating the molecular control of the cell cycle in injury and repair, specifically in the lung. Driscoll joined the USC faculty in 2000 and has maintained a National Institutes of Health R01-funded lab at Children's Hospital for the past 13 years. She was appointed chair of The Saban Research Institute's Institutional Animal Care and Use Committee (IACUC) in 2010 and continues to run an active research program focused on the changes in cellular mechanisms caused by aging and disease.



Jon Thomas PhD, JD

Jon Thomas, PhD, JD, is a co-founding partner at Saybrook Capital, an investment banking and private equity firm based in Santa Monica, Calif. Long interested in the biological sciences, Thomas majored in biology and history at Yale University, where he graduated summa cum laude. As a George C. Marshall Scholar at the University of Oxford, he then earned a doctoral degree with a medical focus in commonwealth history. He subsequently returned to Yale to earn a juris doctor degree at the Yale Law School. While there, Thomas retained an involvement with biology by teaching courses on the legal implications of genetic engineering and the impact of disease on history. Thomas has a longstanding commitment to patient advocacy. He spent more than 15 years on the Board of the Crippled Children's Society of Southern California and served as chair for four years. The organization, now called AbilityFirst, assists children with spinal cord injuries and mental disabilities that could be targets of stem cell therapies. Thomas currently serves as a member of the AbilityFirst Board.



**Scott Fraser, PhD
Keynote Speaker**

Scott Fraser, PhD, is co-director of the Children's Hospital Los Angeles–University of Southern California, Translational Biomedical Imaging Laboratory (TBIL) at The Saban Research Institute of Children's Hospital Los Angeles and provost professor of the Departments of Molecular and Computational Biology and of Biomedical Engineering at USC. Previously, he was the Anna L. Rosen Professor of Biology at the California Institute of Technology (Caltech), where he also founded the Biological Imaging Center in the Beckman Institute in 1991. In addition to his appointments in the Dornsife College of Letters, Arts and Sciences and the Viterbi School of Engineering at USC, Fraser is also active at the Keck School of Medicine of USC and Children's Hospital Los Angeles. Fraser is a world leader in imaging the molecular signals, cell motions and tissue morphogenesis involved in embryogenesis and other complex events using advanced imaging technologies. He completed his undergraduate work at Harvey Mudd College and received his doctorate with distinction in biophysics from The Johns Hopkins University in 1979. Shortly thereafter, he joined the core faculty of the Department of Physiology and Biophysics at the University of California, Irvine, where he rose to the rank of chair, before moving to Caltech in 1991.



**Andrew McMahon, PhD
Keynote Speaker**

Andrew McMahon, PhD, joined USC in July 2012 after 19 years at Harvard College, where he was a chairman of the Department of Molecular and Cellular Biology, a faculty member in the Department of Stem Cells and Regenerative Biology, a founding member of the Harvard Stem Cell Institute and the Frank B. Baird Jr., Professor of Science. McMahon is the Keck provost professor at USC, chair of the Department of Stem Cell Biology and Regenerative Medicine, and director of the Eli and Edythe Broad-CIRM Center for Regenerative Medicine and Stem Cell Biology at the Keck School of Medicine of USC. McMahon is an elected Fellow of the Royal Society, the American Association for the Advancement of Science and the American Academy of Arts and Sciences, and an elected associate member of the European Molecular Biology Organization. The McMahon group's research focuses on the regulatory processes that construct, maintain and repair mammalian organ systems, with a principal focus on the central nervous system, skeleton and kidney.



Laura Perin, PhD

Laura Perin, PhD, is a faculty member of the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute of Children's Hospital Los Angeles and an assistant professor of Surgery in the Division of Urology at the Keck School of Medicine of USC. Her research involves the use of stem cells to restore organ functionality. Prior to joining the faculty at USC, Perin developed her interests in stem cells, regenerative medicine and tissue engineering as a research fellow at Harvard Medical School and as a postdoctoral researcher at the Children's Hospital, California. While at USC, Perin has focused her research on the application of amniotic fluid stem cells and organ progenitors to rescue and restore acute and chronic renal, lung and pancreatic disease phenotypes.



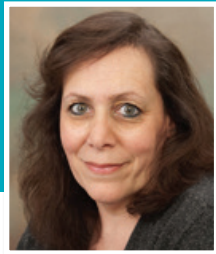
**Lee Niswander, PhD
Keynote Speaker**

Lee A. Niswander, PhD, is professor of Pediatrics and Cell and Developmental Biology at the University of Colorado School of Medicine, section head of Developmental Biology at Children's Hospital Colorado and leader of the Developmental Origins of Health and Disease research emphasis group. She graduated from the University of Colorado-Boulder and received her doctoral degree in genetics from Case Western Reserve University, followed by postdoctoral studies at the University of California, San Francisco. Before returning to Colorado in 2004, she was a member of the Developmental Biology program at Memorial Sloan-Kettering Cancer Center in New York City from 1993-2004. Her honors include a Presidential Early Career Award, a Pew Scholars Award and the Harland Winfield Mossman Developmental Biologist Award, and she has been a Howard Hughes Medical Institute Investigator since 1997. The Niswander lab uses mouse genetics and an understanding of developmental biology to discover genes required for neural tube closure and brain development and their mechanism of action. The laboratory also uses live imaging of tissue development to understand the cellular basis of normal and abnormal development. The goal is to determine the developmental basis of common birth defects and to explore gene-environment interactions that alter the risk of neural tube defects.



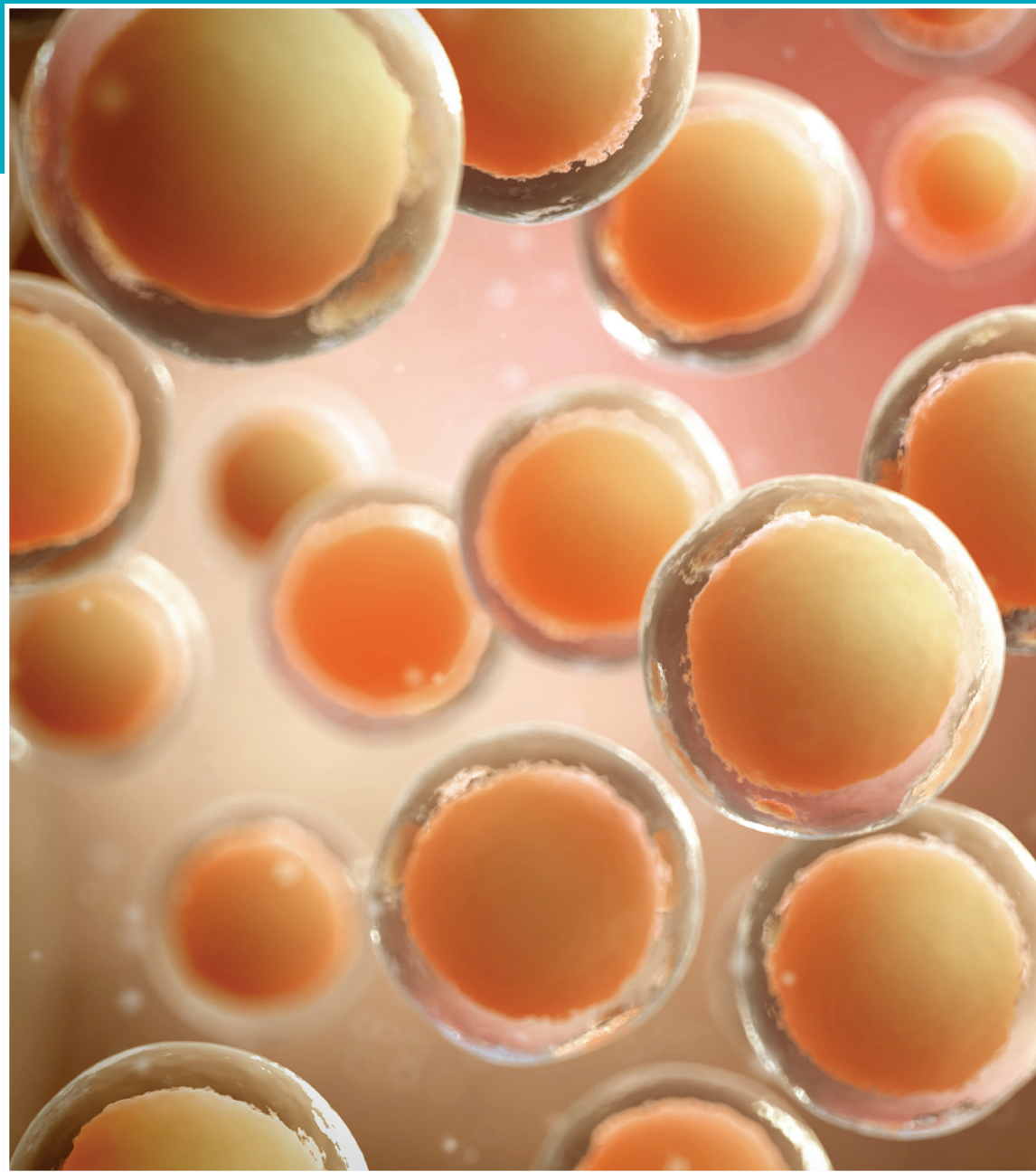
Paula Cannon, PhD

Paula Cannon, PhD, is an associate professor of Microbiology at the Keck School of Medicine of USC, where she leads a research team that studies viruses, stem cells and gene therapy. She obtained her doctoral degree from the University of Liverpool in the United Kingdom, and received postdoctoral training as an HIV scientist at both Oxford and Harvard universities. Although HIV remains the main focus of her work, Cannon also studies highly pathogenic hemorrhagic fever viruses, including Ebola and Lassa fever viruses. Cannon has a longstanding interest in the development of gene therapy as a clinical approach to treating HIV infection, and her recent work in this area is aimed at disrupting the viral co-receptor, CCR5, using zinc finger nucleases (ZFNs). This approach is being developed as a modification to a patient's own hematopoietic stem cells, in an attempt to create a "functional cure" for AIDS patients. Cannon's research is funded by both the National Institutes of Health and the California Institute for Regenerative Medicine.



Karen Aboody, MD

Karen Aboody, MD, received her medical degree at Mount Sinai School of Medicine and her postdoctoral training in molecular neurogenetics at Massachusetts General Hospital, Harvard Medical School. Since 2003, she has headed a translational stem cell oncology laboratory at City of Hope, focused on using tumor-tropic neural stem cells (NSCs) to target therapeutic agents to invasive brain tumors and metastatic cancers. In 2013, she completed a first-in-human safety/feasibility clinical trial for NSC-mediated enzyme/prodrug therapy in recurrent high-grade glioma patients, in collaboration with Rex Moats, PhD, at Children's Hospital Los Angeles and Joseph Frank, MD, MS, at the National Institutes of Health (NIH). Aboody also received first-in-human approval to use Feraheme labeling to track the NSCs by MRI. An \$18 million CIRM Disease Team Award has led to translation of a second-generation, NSC-mediated therapeutic product for brain tumors, with a Phase I trial planned for 2014 in collaboration with Moats and Phil Potter, PhD, of St. Jude Children's Research Hospital. Aboody recently received a \$4.6 million NIH U-01 contract to translate this NSC-mediated therapy for application to pediatric neuroblastoma, a metastatic cancer, with clinical trials planned for 2017. In 2011, she founded TheraBiologics Inc., a clinical stage biopharmaceutical company, to support clinical development of NSC-mediated cancer therapies.

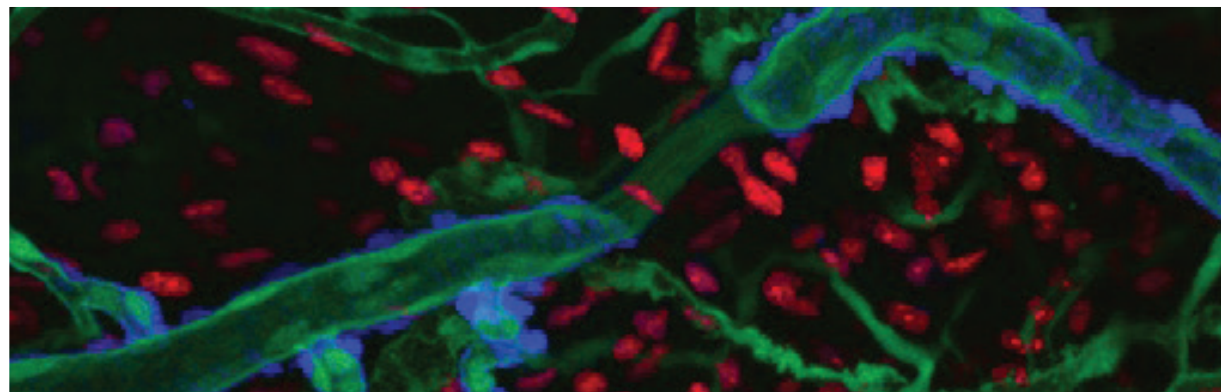


A HISTORY OF RESEARCH AT CHILDREN'S HOSPITAL LOS ANGELES

The research program at Children's Hospital Los Angeles began in 1952. At that time, resources were allocated on a project-by-project basis with equipment being borrowed from clinical laboratories. In the late 1950s, a decision was made to develop a strong basic research program and several highly capable investigators were recruited to lead work in the areas of pathology, hematology-oncology and infectious diseases. During the 1960s those initial areas of inquiry expanded to include endocrinology, virology and genetics. The research program also began getting a national reputation for success at obtaining federal funding. The first endowment was received in 1965. During the next three decades, the program continued to expand its research enterprise with new areas of focus, increases in National Institutes of Health funding and purpose-built research facilities.

Established in 1992, the Research Institute became The Saban Research Institute in 2003 following a \$40 million gift in support of pediatric research made by Cheryl Saban, PhD, Haim Saban and The Saban Family Foundation.

In the course of its evolution, The Saban Research Institute has recruited transformative faculty members dedicated to basic, clinical and translational research in order to further our understanding of the developmental origins of health and disease. Researchers at The Saban Research Institute are committed to the health of the whole child, working in collaborations designed to accelerate the discovery, development and delivery of innovative preventive, diagnostic and treatment options. The Institute works with the local community as well as globally, focusing on individualized health and medicine, the developing mind and regenerative medicine and cellular therapies.



James Terrile – Chair

Bonnie McClure – Vice Chair

Mary Adams O’Connell – Vice Chair

Brooke Anderson

Brian Berliner

Richard D. Cordova, FACHE

Lynda Boone Fetter

Marcia Wilson Hobbs

Jihee Huh

D. Brent Polk, MD

Drew Pomerance

Kathy Luppen Rose

Cheryl Saban, PhD

Elisa Schenkman

Stuart E. Siegel, MD

Victoria Simms, PhD

Erica Van Loon

This image shows a single sheet of white paper with horizontal blue lines, resembling notebook paper. The lines are evenly spaced and run across the width of the page. There is no handwriting or other markings on the paper.



Children's Hospital Los Angeles has been named the best children's hospital in California and among the best in the nation for clinical excellence with its selection to the prestigious U.S. News & World Report Honor Roll. Children's Hospital is home to The Saban Research Institute, one of the largest and most productive pediatric research facilities in the United States. The hospital is also one of America's premier teaching hospitals through its affiliation since 1932 with the Keck School of Medicine of the University of Southern California.

For more information, visit **CHLA.org**. Follow us on **Twitter**, **Facebook**, **YouTube** and **LinkedIn**, or visit our **blog: WeAreChildrens.org**.