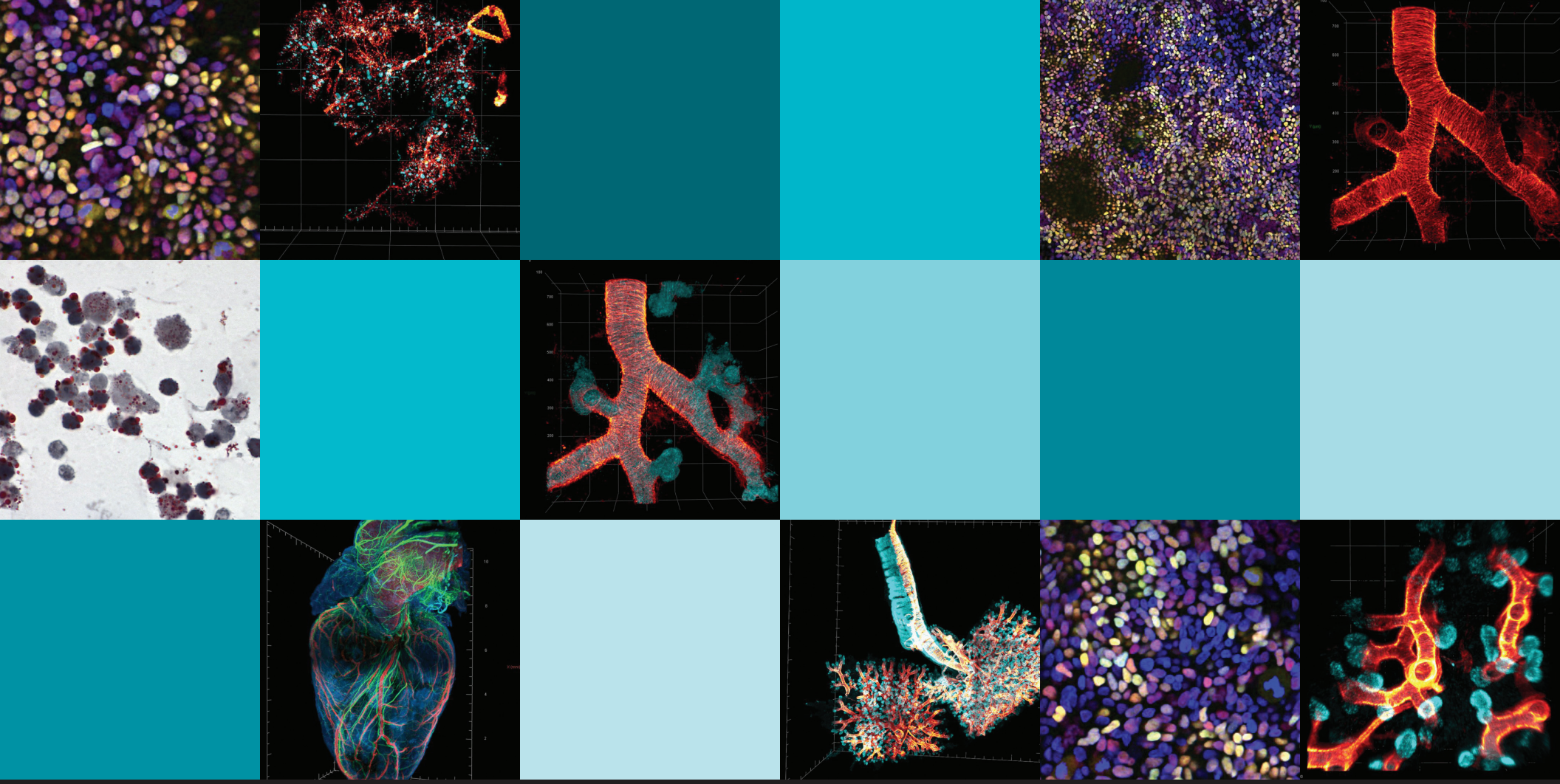


The Saban Research Institute Annual Symposium

MODELS OF HUMAN DISEASE FROM THE LABORATORY TO THE CLINIC

Children's Hospital LOS ANGELES

THE SABAN RESEARCH INSTITUTE

Feb. 1, 2018

USC University of Southern California

WELCOME

Research is fundamental to the mission of Children's Hospital Los Angeles as a pediatric academic medical center. Many of the children cared for at CHLA face extraordinarily complicated health disorders. Although the clinical manifestations of these disorders are many and varied, the methods for studying them—in an effort to identify targetable disease mechanisms that will allow us to develop and deliver the most effective therapies—are quite similar.

Studying organisms that range from single-celled bacteria to zebrafish to small mammals allows researchers to unravel disease processes with the expectation that observations made in one species will provide relevant insights into the biology of another species—specifically, humans. This strategy is fundamental to translational research. The use of model organisms allows investigators access to disease processes in a way that can be experimentally manipulated while protecting patients from early and repetitive testing. The vast majority of these studies could never be done in humans, for both practical and ethical reasons.

While we endeavor to protect patients and conduct relevant and transformative research using a model system, challenging questions remain, such as: Which is the most appropriate model for answering a specific, biological question? Why do some model systems lead to tremendous advances in understanding that result in the development of new treatments in humans, while others do not?

To attempt to answer these questions, we have collaborated with our academic partner, the University of Southern California, to assemble an esteemed group of investigators who grapple daily with the quest to move great ideas from the bench to the bedside. Through their presentations and the ensuing dialogue and debate, we will have an opportunity to gain new insights, form new collaborations, and ultimately advance our efforts to improve patients' lives.

For their guidance in planning today's symposium, we would like to thank Mark Frey, PhD, Robert Seeger, MD, and Andrew McMahon, PhD. We would also like to acknowledge The Saban Research Institute staff members for their efforts in creating this event.

We extend our gratitude to the philanthropists—those who are with us today as well as those who were unable to attend—who support our research. Thank you for your vision and commitment. Finally, we would like to acknowledge Cheryl and Haim Saban and The Saban Family Foundation for their ongoing support.

Paul S. Viviano
President and Chief Executive Officer
Children's Hospital Los Angeles

Bradley S. Peterson, MD
Interim Director, The Saban Research Institute
Director, Institute for the Developing Mind

SYMPOSIUM OVERVIEW

Models of Human Disease From the Laboratory to the Clinic

Preclinical models of disease are an essential element of the scientific effort to improve human health. Without an experimental system that reflects the complex biochemical and biological interactions within a tissue—and the defects in these that drive disease—a researcher is unable to test the likely effectiveness of a new drug or therapeutic method. Similarly, basic discovery of pathogenic mechanisms requires the use of experimental models of the target disease.

Preclinical models are not, of course, all created equal. By definition, they are approximations of the human biological system of interest. In choosing or developing a model system, an investigator must weigh multiple variables, including cost, difficulty, complexity (ranging from cells in a dish all the way to in vivo studies in intact organisms), and predicted fidelity to human biology. The "correct" choice is often imperfect, and tends to be empirical and context-dependent. Using a good model system for a study is essential, however, and can make or break the effort to translate a great idea from the bench to the bedside.

Our 2018 speakers at The Saban Research Institute Symposium will discuss how, across a broad range of human health research topics, the choice of an effective model system has been critical for progress. Presentations will highlight successes and failures of models in driving translatable discovery, and the considerations that made the difference. In other words: what works, what doesn't, and why.

Symposium Co-organizer:
Mark R. Frey, PhD

Mark R. Frey, PhD, of the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute, is an associate professor of Pediatrics and of Biochemistry and Molecular Medicine at the Keck School of Medicine of the University of Southern California (USC). He is a basic and early translational researcher focused on epithelial biology and intestinal disorders, an interest he has maintained since his doctoral work at the State University of New York at Buffalo. There, he helped define the role of protein kinase C isoforms in driving cell-cycle arrest and repressing tumorigenesis in the gut. After postdoctoral work at Vanderbilt University on EGF-stimulated intestinal restitution, Frey was recruited to USC and CHLA in 2010.

Research in Frey's lab is primarily focused on the role of neuregulin-family growth factors and their receptors (ErbB3 and ErbB4) in promoting intestinal repair and regeneration after injury or inflammation. His group was the first to report a functional role for ErbB4 in the gut, showing that it is induced by injury as a compensatory response to limit inflammation and prevent barrier breach. This work showed that activating ErbB4 with an exogenous ligand is protective in preclinical models of inflammatory bowel disease and necrotizing enterocolitis. Frey currently serves as vice-chair for the Growth, Development and Child Health section of the American Gastroenterological Association, as the co-chair for the American Physiological Society GI and Liver Programming Committee for the annual Experimental Biology meeting, and as a senior editor for Experimental Physiology.

Symposium Co-organizer:
Andrew P. McMahon, PhD

Andrew (Andy) McMahon is chair of the Department of Stem Cell Biology and Regenerative Medicine and director of the Eli and Edythe Broad Center for Regenerative Medicine and Stem Cell Research within the Keck School of Medicine of USC. McMahon joined USC from Harvard University in 2012 as the W.M. Keck Provost Professor, establishing the current department.

McMahon's research has provided fundamental insights into the cell interactions driving assembly of multiple organ systems. His current research focuses on development, injury and repair of the mammalian kidney.

McMahon received his bachelor's degree from Oxford University and his doctorate from University College London. After a postdoctoral period at the California Institute of Technology, McMahon started his independent research career at the National Institute for Medical Research, London. In 1988, McMahon took up a position as assistant member at the Roche Institute for Molecular Biology, becoming full member and chair of the department in 1992. In 1993, he joined the faculty at Harvard University as a professor of Molecular and Cellular Biology. In a 19-year career at Harvard, McMahon was the Frank B. Baird Jr. Professor of Science, chair of the Department of Cell and Developmental Biology, a founding faculty of the Department of Stem Cells and Regenerative Biology, and principal investigator of the Harvard Stem Cell Institute.

McMahon is an elected fellow of the American Association for the Advancement of Science, the American Academy of Arts and Sciences, the European Molecular Biology Organization and the Royal Society.

Symposium Co-organizer:
Robert C. Seeger, MD, MS

Robert C. Seeger, MD, MS, is a professor of Pediatrics at the Keck School of Medicine of USC and former director of Translational and Basic Science at the Children's Center for Cancer and Blood Diseases and leader of the Cancer and Blood Diseases Research Program at The Saban Research Institute. He currently is an associate director of The Saban Research Institute.

Few have had as profound an impact on the understanding and treatment of a single type of cancer as Seeger has had on neuroblastoma. By dedicating his career to laboratory and clinical investigations into this disease, Seeger has led the way to a better understanding of the biology of neuroblastoma, improved risk assessment and monitoring of patients, and increased survival of children with the high-risk form of the disease. He has been involved in every major medical advancement in treating this malignancy, including playing a key role in developing dinutuximab, which in 2015 became the first therapy specifically aimed at high-risk neuroblastoma to receive approval from the U.S. Food and Drug Administration. In recognition of Seeger's groundbreaking contributions, the Advances in Neuroblastoma Research Association and the Pediatric Blood and Marrow Transplant Consortium honored him with Lifetime Achievement Awards in 2016.

Currently, Seeger is co-principal investigator of a National Institutes of Health/National Cancer Institute-funded Program Project Grant and a Department of Defense Translational Team Science award. He is principal investigator on five studies funded by foundations and one supported by a pharmaceutical company. Seeger co-founded the New Approaches to Neuroblastoma Therapy consortium (see www.nant.org), which develops new treatments for patients with refractory/relapsed high-risk neuroblastoma.

SCHEDULE

8:15 – 9 a.m.
Continental Breakfast and Check-in
The Saban Research Building lobby

9 – 9:15 a.m.
Symposium Welcome
Bradley S. Peterson, MD, The Saban Research Institute of Children's Hospital Los Angeles; Keck School of Medicine of the University of Southern California (USC)

Paul S. Viviano, President and Chief Executive Officer, Children's Hospital Los Angeles

Randolph W. Hall, PhD, Vice President of Research; Daniel J. Epstein Department of Industrial and Systems Engineering, USC

SESSION 1

9:15 – 10:05 a.m.
The Biology of Memory and Age-Related Memory Loss
Eric R. Kandel, MD, Columbia University

10:05 – 10:25 a.m.
Mitochondrial-Derived Peptides
Pinchas Cohen, MD, Leonard Davis School of Gerontology, USC

10:25 – 10:45 a.m.
Using Patient-Derived Disease Models to Develop Effective Treatment Strategies for ALS
Justin Ichida, PhD, Keck School of Medicine of USC

10:45 – 11 a.m.
Break

SESSION 2

11 – 11:50 a.m.
Deconstructing Pancreatic Development and Disease With Human Pluripotent Stem Cells
Maike Sander, MD, University of California, San Diego

11:50 a.m. – 12:10 p.m.
Precise Gene Engineering of Blood Stem Cells
Paula Cannon, PhD, Keck School of Medicine of USC

12:10 – 12:30 p.m.
From Bedside to Bench and Back to Bedside: Moving Forward With Gene Editing and Autologous Stem Cell Transplantation
Senta K. Georgia, PhD, The Saban Research Institute of CHLA; Keck School of Medicine of USC

12:30 – 1:30 p.m.
Lunch
Anita S. Watson Courtyard of The Saban Research Building

SESSION 3

1:30 – 2:20 p.m.
Employing Humanized Mouse Models to Study IBD Pathogenesis and the Development of Novel Therapeutics
Scott B. Snapper, MD, PhD, Harvard Medical School

2:20 – 2:40 p.m.
Exosomal MicroRNAs Shape the Biology of the Tumor Microenvironment
Muller Fabbri, MD, PhD, The Saban Research Institute of CHLA; Keck School of Medicine of USC

2:40 – 3 p.m.
Injury and Repair: What a Mouse Can Teach Us About Human Intestinal Health
D. Brent Polk, MD, The Saban Research Institute of CHLA; Keck School of Medicine of USC

3 – 3:15 p.m.
Break

3:15 – 4:05 p.m.
The Ascent of CAR-T Cells for Pediatric Leukemia: From Models to Trials to an FDA-Approved Drug
Michael C. Jensen, MD, University of Washington School of Medicine

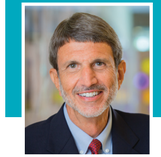
4:05 – 4:20 p.m.
Closing Remarks
Robert E. Shaddy, MD, The Saban Research Institute of CHLA; Keck School of Medicine of USC

4:20 – 5 p.m.
Reception
Anita S. Watson Courtyard of The Saban Research Building

Bradley S. Peterson, MD

Bradley S. Peterson, MD, is the interim director of The Saban Research Institute and director of the Institute for the Developing Mind at Children's Hospital Los Angeles. He is also vice chair for Research and director of Child and Adolescent Psychiatry in the Department of Psychiatry, and a professor at the Keck School of Medicine of the University of Southern California (USC). He received his bachelor's degree and graduated summa cum laude from Tulane University and received his doctorate of medicine from the University of Wisconsin-Madison. He trained in general psychiatry at Massachusetts General Hospital and Harvard University, in child psychiatry at the Child Study Center of Yale University, and in psychoanalysis at the Western New England Institute of Psychoanalysis. He was previously a faculty member at the Yale Child Study Center and then at Columbia University, where he was the founding director of MRI Research and the director of Child and Adolescent Psychiatry.

Peterson's research uses brain imaging technologies to understand the origins of neuropsychiatric disorders by mapping the constitutional and environmental influences that confer risk for illness or protect against it, trigger its onset or progression, compensate for its presence, or mediate effective treatments. He has published more than 300 peer-reviewed papers and 30 book chapters, and has mentored a dozen graduate and medical students and 50 postdoctoral fellows and junior research faculty members.



Paul S. Viviano

Paul S. Viviano is a health care leader who has directed academic, nonprofit and for-profit health care organizations that deliver excellence in clinical care, research and diagnostics for three decades. Paul joined Children's Hospital Los Angeles as president and chief executive officer in 2015 and serves as a member of the institution's Board of Trustees.

Previous to serving as president and chief executive of CHLA, Paul served as the CEO and associate vice chancellor for the UC San Diego Health System, an institution noted for its leadership in medical research and patient care. He has also served as the president and chief executive officer of University of Southern California (USC) University Hospital and USC/Norris Cancer Hospital and held various chief executive roles within the St. Joseph Health System, ultimately serving as the president and chief operating officer for the system. He served as chairman of the board and CEO of Alliance HealthCare Services, the nation's largest provider of outpatient diagnostic imaging services and radiation oncology services, for 10 years.

Paul currently serves on the boards of several organizations, including as chair of the California Children's Hospital Association. He is also a member of the Public Policy Committee for the national Children's Hospital Association and chair of the Board of Trustees for Loyola Marymount University. He also is a board member for Alliance HealthCare Services.

Randolph W. Hall, PhD

Randolph Hall, PhD, is the vice president of Research at the University of Southern California, where he oversees research advancement, administration and ethics. Hall's experience includes serving as the founder/principal investigator for two national research centers, the Center for Risk and Economic Analysis of Terrorism Events (CREATE), and the National Center for Metropolitan Transportation Research (METRANS). He also served as senior associate dean for research in the Viterbi School of Engineering and chair of Industrial and Systems Engineering when it became the first named academic department at the University of Southern California, upon receipt of a $10 million gift from Daniel J. Epstein. He is the author of several books, and is editor for Patient Flow, Reducing Delay in Healthcare Delivery and the Handbook of Healthcare System Scheduling.

Hall has led the creation of policies to catalyze collaborative research, including changes in promotions and tenure, research attribution and shared repositories; creation of funding programs that support collaborative research and shared equipment; and infrastructure and events enabling digital scholarship, as well as standards for research transparency and reproducibility. He has helped faculty create national research centers, built alliances with external research institutes, developed the Center for Excellence in Research, created the Washington, D.C.-based research advancement office, and built an integrated research office that encompasses contracts and grants, technology transfer, human subject protection, animal resources, research ethics, research training, research advancement and internal grant programs. Hall serves on the boards for Keck Medicine and the Alfred Mann Institute at USC, is chair of the board for the University Industry Demonstration Partnership and is on the Executive Committee for the Southern California Innovation Alliance.

Eric R. Kandel, MD

Eric R. Kandel, MD, is the Kavli Professor at Columbia University and director of the Kavli Institute for Brain Science. He is also co-director of the Mortimer B. Zuckerman Mind Brain Behavior Institute and an investigator at the Howard Hughes Medical Institute. A graduate of Harvard College and NYU School of Medicine, Kandel trained in neurobiology at the National Institutes of Health (NIH) and in psychiatry at Harvard Medical School. He joined the faculty of the College of Physicians and Surgeons at Columbia University in 1974. At Columbia, Kandel organized the neuroscience curriculum. He is an editor of "Principles of Neural Science," the standard textbook in the field now in its fifth edition. His previous book on art, "The Age of Insight: The Quest to Understand the Unconscious in Art, Mind, and Brain From Vienna 1900 to the Present," won the Kreisky Award in Literature, Austria's highest literary award. Kandel's new book, entitled "Reductionism in Art and Brain Science: Bridging the Two Cultures," published by Columbia University Press, has just been released.

Kandel's research has been concerned with the molecular mechanisms of memory storage in Aplysia and mice. Recently, he has studied age-related memory disorders; post-traumatic stress disorders; and nicotine, alcohol, marijuana and cocaine addiction.

Kandel has received 23 honorary degrees. He has been recognized with the Albert Lasker Award, the Heineken Award of the Netherlands, the Gairdner Award of Canada, the Harvey Prize and the Wolf Prize of Israel, the National Medal of Science in the United States, and the Nobel Prize for Physiology or Medicine in 2000.

Pinchas Cohen, MD

Pinchas Cohen, MD, is the dean of the Leonard Davis School of Gerontology and executive director of the Andrus Gerontology Center at USC. He graduated with highest honors from the Technion American Medical School in Israel, trained at Stanford University, and has held faculty positions at the University of Pennsylvania and the University of California, Los Angeles (UCLA). Cohen has received numerous awards for his research, including a National Institute on Aging "EUREKA" prize, a Transformative Research R01 award from the director of the NIH, and an award from the Glenn Foundation for Medical Research. He pioneered the emerging science of mitochondrial-derived peptides and holds several patents for these novel hormones. He is a co-founder and director of CohBar, a biotechnology company developing treatments for diseases of aging. Cohen serves on the boards of several professional journals and societies, including the American Society of Aging and the Milken Institute Center for the Future of Aging, and has been president of the Growth Hormone Research Society. Cohen is currently leading several new initiatives at the USC Davis School, including the Lifespan Health Initiative, the development of a Center for Digital Aging, and the creation of tools for "Personalized Aging," using the latest technologies such as genomics to individualize healthy-aging strategies.

Justin Ichida, PhD

Justin Ichida, PhD, is an assistant professor in the Department of Stem Cell Biology and Regenerative Medicine at the Keck School of Medicine of USC. He is also a New York Stem Cell Foundation Robertson Investigator. He completed his doctorate at Harvard Medical School in the laboratory of Jack Szostak, PhD, and was a postdoctoral fellow in the laboratory of Kevin Eggan, PhD, in the Department of Stem Cell and Regenerative Biology at Harvard University. Ichida's current research is focused on using patient-specific disease models to define the mechanisms that lead to neuronal loss in ALS and frontotemporal dementia.

Maike Sander, MD

Maike Sander, MD, is the director of the Pediatric Diabetes Research Center and co-director of the Center on Diabetes in the Institute of Engineering in Medicine at University of California, San Diego (UCSD). The major focus of her research is to understand the molecular mechanisms that control the formation and proper function of insulin-producing pancreatic beta cells. Her work combines genetic approaches in mice and human embryonic stem cells with next-generation sequencing-based assays. By uncovering fundamental mechanisms of cell fate determination and plasticity in the context of beta cell development, regeneration and pathogenesis of diabetes, her work aims to develop novel therapeutic strategies for diabetes. Sander obtained a medical degree from the University of Heidelberg in Germany and held faculty positions at the University of Hamburg, Germany, and the University of California, Irvine, before moving to UCSD in 2008. She is an elected member of the American Society of Clinical Investigation and the German Academy of Sciences (Leopoldina), and a member of the NIH-Human Islet Research Network. She is the recipient of the Grodsky Award from the Juvenile Diabetes Research Foundation and the 2017 Humboldt Research Award.

Paula Cannon, PhD

Paula Cannon, PhD, is a professor at the Keck School of Medicine of USC. She studies genome engineering in hematopoietic stem cells, with an emphasis on developing new therapies for HIV/AIDS. Cannon earned her doctorate from the University of Liverpool and did postdoctoral training at Harvard and Oxford Universities. In 2010, her team was the first to develop genome-engineering tools to knock out the CCR5 gene in human hematopoietic stem cells, which has now led to a clinical trial in HIV-infected individuals. She continues to develop platforms that promote high levels of precise genome editing in human cells, which is extending the applications of this technology for genetic diseases of the blood and immune systems.

Senta K. Georgia, PhD

Senta K. Georgia, PhD, is a researcher at the Center for Endocrinology, Diabetes and Metabolism at CHLA and the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute. She is also an assistant professor in the Departments of Pediatrics and Stem Cells and Regenerative Medicine at the Keck School of Medicine of USC. Her lab at CHLA focuses on three approaches to insulin-cell regeneration: (1) inducing replication of existing insulin cells; (2) using epigenetic manipulation to induce cellular reprogramming into an insulin cell fate; and (3) understanding mechanisms that govern human insulin cell differentiation to make new insulin cells for patients who have diabetes. Georgia attended Stanford University, earning her Bachelor of Science degree in biological sciences, with a minor in philosophy and dual departmental honors in biological sciences and ethics in society. She earned her doctorate at UCLA in molecular biology and was a postdoctoral fellow and assistant adjunct professor at the Hillblom Islet Research Center at UCLA.

Scott B. Snapper, MD, PhD

Scott B. Snapper, MD, PhD, professor of Medicine at Harvard Medical School, is the Wolpow Family Chair and director of the Center for Inflammatory Bowel Disease at Boston Children's Hospital. He is also the director of IBD Research within the Gastroenterology Division at Brigham & Women's Hospital. After graduating summa cum laude from Tufts University with a Bachelor of Science degree in chemistry and biology in 1983, Snapper pursued medical studies and doctoral research at Albert Einstein College of Medicine, where he fell in love with microbiology and immunology and received his medical degree and doctorate in 1990.

Snapper's laboratory is investigating how a defective immune system can contribute simultaneously to immunodeficiency and intestinal inflammation with some focus on human immunodeficiencies. The lab has made numerous key discoveries related to patients with Wiskott-Aldrich syndrome and, more recently, to those with IL-10 receptor deficiency. Currently, his laboratory is characterizing new genes that are associated with inflammatory bowel disease (IBD) and testing novel treatment strategies to manipulate immunoregulatory circuits in mice and humans for IBD prevention and therapy.

Snapper has authored over 125 articles and book chapters, and has received multiple grants from the NIH, the Crohn's and Colitis Foundation, the Helmsley Charitable Trust and pharmaceutical companies. He is on the editorial board of several journals, is a member of numerous scientific advisory boards of pharmaceutical and biotechnology companies, serves as a permanent member of the Gastrointestinal Mucosal Pathobiology Study Section of the NIH, and is the chair of the National Scientific Advisory Committee of the Crohn's & Colitis Foundation.

Muller Fabbri, MD, PhD

Muller Fabbri, MD, PhD, of the Children's Center for Cancer and Blood Diseases and The Saban Research Institute of Children's Hospital Los Angeles, is an assistant professor of Pediatrics and Molecular Microbiology and Immunology at the Keck School of Medicine of USC. Fabbri's lab at CHLA is focused on studying how microRNAs (and other non-coding RNAs) communicate between cancer cells and the surrounding cells in the tumor microenvironment and how this affects cancer biology and the development of resistance to chemo- and radiotherapy.

Fabbri has provided the first evidence of the existence of epi-miRNAs, and has described a complex miRNA-based network responsible for the prognostic significance of chromosomal aberrations in chronic lymphocytic leukemia. He has also contributed to the identification of the role of miRNAs in mismatch repair mechanisms in cancer. More recently, Fabbri has identified a completely new mechanism of action for miRNAs as ligands of Toll-like receptors, providing the first evidence that miRNAs can function in a ligand-receptor fashion, similar to hormones.

Fabbri is the author of more than 70 peer-reviewed publications, has authored several book chapters and serves on the editorial board of six international scientific journals.

D. Brent Polk, MD

D. Brent Polk, MD, of the Developmental Biology and Regenerative Medicine Program at The Saban Research Institute of Children's Hospital Los Angeles, is a professor of Pediatrics and Biochemistry and Molecular Medicine at the Keck School of Medicine of USC. He formerly served as chair of the Department of Pediatrics, physician in chief, vice president for Academic Affairs and director of The Saban Research Institute of CHLA, and as chair of Pediatrics and vice dean for Child Health at the Keck School of Medicine.

Polk's research focuses on the regulation of growth and development of the intestine, making important contributions to our understanding of the relationship between inflammation and tumorigenesis. Polk is a fellow of the American Gastroenterological Society, the American Association for the Advancement of Science, the Royal College of Physicians of Edinburgh and the Association of American Physicians. He is a member of several professional organizations, including the American Pediatric Society, the Crohn's and Colitis Foundation, the American Society for 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.

Polk previously served as chief of the D. Brent Polk Division of Pediatric Gastroenterology, Hepatology and Nutrition, director of the Digestive Disease Research Center and a tenured professor of Pediatrics and Cell and Developmental Biology at Vanderbilt University Medical Center. He received a bachelor's degree in biology and chemistry from Ouachita University in Arkadelphia, Arkansas, and his medical degree from the University of Arkansas for Medical Sciences.

Michael C. Jensen, MD

Michael C. Jensen, MD, is director of the Ben Towne Center for Childhood Cancer at Seattle Children's Research Institute and professor of Hematology-Oncology at the University of Washington School of Medicine. He holds the Janet and Jim Sinegal Endowed Chair in Childhood Cancer Research. Jensen graduated from the University of Pennsylvania School of Medicine and completed training in pediatric hematology and oncology at the University of Washington/Fred Hutchinson Cancer Research Center (FHCRC). Under the mentorship of Philip Greenberg, MD, Jensen focused on the immunobiology of tumor-specific T-cells. Following completion of his fellowship, he joined the faculty at City of Hope National Medical Center, where he built a translational research program integrating gene therapy and cellular immunotherapy for cancer. This program grew into the Department of Cancer Immunotherapeutics and Tumor Immunology within the Beckman Research Institute and was incorporated into the institution's NCI-Comprehensive Cancer Center as the Cancer Immunotherapeutics Program, with Jensen as its leader.

During his tenure at City of Hope, Jensen's research program placed a strong emphasis on bench-to-bedside translational research and resulted in five FDA-authorized Investigational New Drug Applications covering first-in-human applications of adoptive transfer of genetically engineered CAR T-cells. In 2010, he joined the University of Washington School of Medicine faculty and serves as associate director and program head for the UW-FHCRC Cancer Consortium. Together with colleagues at FHCRC and Memorial Sloan Kettering Cancer Center, Jensen is a scientific founder of Juno Therapeutics, Inc., a Seattle-based biotech startup focused on engineered T-cell cancer therapeutics.

Robert E. Shaddy, MD

Robert E. Shaddy, MD, is pediatrician in chief and senior vice president of Academic Affairs at Children's Hospital Los Angeles, and chair of the Department of Pediatrics at the Keck School of Medicine of USC. In these roles, he provides oversight for faculty appointments as well as educational and research activities including those performed at The Saban Research Institute. In his role as chair of Pediatrics, Shaddy oversees department faculty at CHLA and LAC+USC Medical Center campuses.

Prior to his tenure at CHLA and USC, he served as vice chair of the Department of Pediatrics at the Perelman School of Medicine at the University of Pennsylvania, and as the chief of the Division of Cardiology and the Jennifer Terker Endowed Chair in Pediatric Cardiology at Children's Hospital of Philadelphia.

Shaddy is recognized internationally for his research into pediatric heart failure and heart transplantation. His expertise also includes the use of echocardiography and other imaging in pediatric heart patients, the use of ventricular assist devices in pediatric patients, and the effectiveness of pharmaceutical drugs in treating heart failure in children.

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 its success at obtaining federal funding, and it received its first endowment in 1965. During the next three decades, the institution 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.

THE SABAN RESEARCH INSTITUTE COMMITTEE 2017

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Children's Hospital LOS ANGELES®

THE SABAN RESEARCH INSTITUTE

Children's Hospital Los Angeles has been ranked the top 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 of children's hospitals for 2017-18. CHLA 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 hospital blog, **CHLA.org/BLOG**, and our research blog: **ResearCHLAblog.org**.

Cover image credit: G. Esteban Fernandez, PhD, of the Cellular Imaging Core. The images represent the work of several investigators, including Denise Al Alam, PhD; Senta Georgia, PhD; Richard Kim, MD; and Yong-Mi Kim, MD, PhD, MPH.