Duke | MARGOLIS CENTER *for* Health Policy

Advancing Endpoint Development for Preterm Neonates with Pulmonary Morbidities

The JW Marriott • Washington, DC
October 2, 2018

Speaker Biographies

Judy Aschner is Professor of Pediatrics, and of Obstetrics, Gynecology and Woman's Health at the Albert Einstein College of Medicine in the Bronx, NY. Dr. Aschner completed her medical school training, pediatric residency and neonatology fellowship at the University of Rochester School of Medicine. She was recruited to Einstein/Montefiore from Vanderbilt University where she was the Julia Carell Stadler Professor of Pediatrics and Chief of the Mildred Stahlman Division of Neonatology from 2004-2013. From 2013-2018 she was the Michael I. Cohen Professor and University Chair of Pediatrics and Physician-in-Chief at the Children's Hospital at Montefiore. Dr. Aschner has mentored the next generation of neonatologists and pediatricians and partnered with institutions across the United States and around the world to improve the outcomes of pregnancy and the health outcomes of infants born prematurely. Her NIH-funded translational research program focuses on (a) novel therapies to prevent and treat neonatal lung diseases and (b) understanding and mitigating the impact of environmental exposures in infancy on health outcomes for infants and children. Dr. Aschner has held a number of leadership roles in state and national organizations, including Secretary-Treasurer of the American Pediatric Society and Chair of the Organization of Neonatology Program Directors. She currently is the Executive Director of the Federation of Pediatrics Organizations (FOPO). On the international scene, she is Vice-Chairperson and member of the Board of Directors of IPOKRaTES Foundation, an organization dedicated to continuing medical education in the developing world. She is a member of the Board of External Experts (BEE) at the National Heart, Lung and Blood Institution of the NIH, and is a member of the National Board of Trustees of the March of Dimes. She lectures widely on topics related to neonatal lung disease and environmental health in the US and abroad.

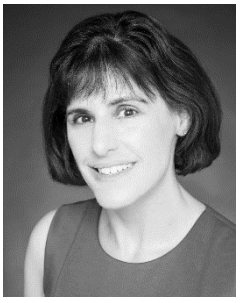
Gerri Baer is a Medical Officer and Team Leader for Neonatology in the Office of Pediatric Therapeutics. She completed pediatric residency and chief residency at Mount Sinai Medical Center in New York City. After residency she worked as a NICU hospitalist for a year before starting her neonatology fellowship training at the Children's Hospital of Philadelphia. She is board-certified in pediatrics and neonatal-perinatal medicine. Prior to joining the FDA in September of 2015, she worked for 8 years as an attending neonatologist at Holy Cross, which has a Level III NICU in Silver Spring, Maryland. She was a partner in the practice and site lead for the Vermont-Oxford Very Low Birth Weight Neonatal Database. At FDA, she has established the Neonatal-Perinatal Medicine consultation service and is involved with neonatal product development across the Centers. She also serves on the coordinating committee of the International Neonatal Consortium (INC) and represents the FDA on several INC working groups.

Roberta Ballard is Emeritus Professor of Pediatrics at the University of California, San Francisco, and Emeritus Professor and of Pediatrics and Ob/Gyne at the University of Pennsylvania and the Children's Hospital of Philadelphia. Roberta received her MD at the University of Chicago and her residency training in pediatrics at the University of Chicago and Stanford and fellowship in Neonatal Perinatal medicine at George Washington University Hospital and the Cardiovascular Research Institute (CVRI) at UCSF. In 1972 Roberta became Chief of the NICU at Mt. Zion Hospital San Francisco and later became Chief of the Pediatric program and pediatric residency and developed a neonatal fellowship training program in collaboration with UCSF and the CVRI over the next 19 years. Subsequently she, along with her husband Phil Ballard MD PhD led the neonatology program at the Children's Hospital of Philadelphia (CHOP) and the University of Pennsylvania for 15 years as Chief and Director of Research, respectively. They were jointly awarded the PAS Maureen Andrew Mentoring award in 2013 in recognition of mentoring activities over their careers. Roberta has been involved in developing and directing all aspects of NIH-funded multicenter clinical trials in the neonate over more than 25 years and has been funded for multicenter randomized clinical trials (RCTs) for the prevention of the Chronic Lung Disease of prematurity (Bronchopulmonary Dysplasia, BPD) since 1991. She was PI of the NICHD-funded antenatal TRH trial which demonstrated that the addition of TRH to antenatal glucocorticoid does not improve respiratory outcome, ending use of that combination in perinatal practice. Subsequently she was PI of the NHLBI-funded NO CLD trial, which demonstrated a dosing approach for inhaled Nitric Oxide (iNO) therapy that is safe and effective in improving survival without BPD in some high risk ventilated infants and improving their pulmonary outcome through 1 year of age. She was PI of a NHLBI funded multicenter trial (25 sites) examining the combination of iNO with late doses of surfactant (TOLSURF) and assessing pulmonary and neurodevelopmental outcome through age 2. She is currently involved in a pilot dose escalation study of budesonide in surfactant to prevent BPD. Her research has been done as "bench to bedside" work with her collaborator of more than 40 years – Dr. Philip L. Ballard

Gregory Daniel is a Clinical Professor in Duke's Fuqua School of Business and Deputy Director in the Duke-Robert J. Margolis Center for Health Policy at Duke University. Dr. Daniel directs the DC-based office of the Center and leads the Center's pharmaceutical and medical device policy portfolio which includes developing policy and data strategies for improving development and access to innovative pharmaceutical and medical device technologies. This includes post-market evidence development to support increased value, improving regulatory science and drug development tools, optimizing biomedical innovation, and supporting drug and device payment reform. Dr. Daniel is also a Senior Advisor to the Reagan-Udall Foundation for the FDA and Adjunct Associate Professor in the Division of Pharmaceutical Outcomes and Policy at the UNC Eshelman School of Pharmacy. Previously, he was Managing Director for Evidence Development & Biomedical Innovation in the Center for Health Policy and Fellow in Economic Studies at the Brookings Institution and Vice President, Government and Academic Research at HealthCore (subsidiary of Anthem, Inc). Dr. Daniel's research expertise includes utilizing electronic health data in designing research in health outcomes and pharmacoeconomics, comparative effectiveness, and drug safety and pharmacoepidemiology. Dr. Daniel received a PhD in pharmaceutical economics, policy and outcomes form the University of Arizona, as well as an MPH, MS, and BS in Pharmacy all from The Ohio State University.

Jonathan Davis is Vice-Chair of Pediatrics, Chief of Newborn Medicine, and the Associate Director of the Clinical and Translational Science Institute at the Floating Hospital for Children at Tufts Medical Center and Professor of Pediatrics at Tufts University School of Medicine. His research has focused on neonatal drug development for the prevention and treatment of a variety of neonatal conditions. He has authored approximately 200 manuscripts and book chapters and received numerous grant awards from the NIH, the FDA, the March of Dimes, and many others. He is currently funded by NIH and FDA to develop better outcome measures for clinical trials and new and existing therapeutics to improve neonatal outcome. Dr. Davis is Chair of the Neonatal Advisory Committee in the Office of the Commissioner at FDA, the Director of the International Neonatal Consortium, and a member of Leadership Council of the American Pediatric Society. These positions permit him to work closely with governments, academic leaders, industry and families to promote the development of important therapeutics for infants and children.

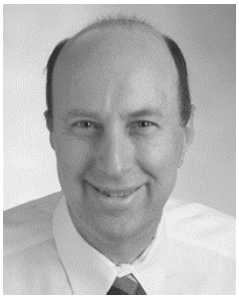
Jennifer Degl is the mother of four, including a micro preemie who was born at 23 weeks gestation, and the founder of Speaking for Moms and Babies, Inc. She is also the author of two books, a member of The International Neonatal Consortium, NIDCAP, the Preemie Parent Alliance and she serves on The Board of Directors of The Morgan Leary Vaughan Fund, which is dedicated to preventing Necrotizing Enterocolitis in premature babies. Jennifer is also a writer for Huffington Post Parents and The Mighty and has spoken on Capitol Hill on two different occasions to promote legislation on behalf of premature babies. You can learn more about Jennifer at http://www.speakingformomsandbabies.com.



Deb Discenza, the mother to a former preemie, supports, educates, and advocates for families in the NICU through PreemieWorld. She also educates professionals through articles, media interviews and speeches. She is the head of the global Inspire Preemie Community and the co-author of "The Preemie Parent's Survival Guide to the NICU."

Wakako Eklund has been working with neonates since the mid 1990's when she made a drastic switch from the adult critical care world to NICU. She later became neonatal NP and further received her doctorate in related area. She lost a prematurely born 34 week cousin in her early years to RDS, NEC, DIC before the time of antenatal steroids or surfactant. The suffering she witnessed in her family heavily weight upon her but propels her to do something to ensure that today's newborns have more options. She is honored to be invited to today's event to contribute in a small way.

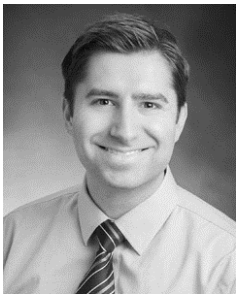
Laura Fabbri is an Italian biologist with a PhD in clinical pharmacology, working in Pharma companies for 30 years. Her experience as clinical research manager started at GlaxoWellcome (now GSK), in 1988 in neurology / psychiatry / endocrinology; she worked in the Italian affiliate and in corporate (London) for 1 year contributing to the launch on the market of the first triptan for migraine, which was sumatriptan (Imigran®). In 2001 Laura was employed by Chiesi Pharmaceutical, corporate, firstly as neurology & psychiatry area manager, becoming then head of clinical neonatology 10 year ago; in this role she is responsible of Curosurf® life cycle management and of clinical development of new neonatal and pediatric products. She coordinates different clinical team groups to deliver clinical projects on phase I-IV drugs, nationally and worldwide; she writes clinical development plans and single clinical protocols; suggests case record forms design; writes study reports and manuscripts for national and international journals; sometime presents data at congresses. She continuously gives scientific support to the Regulatory Affairs (briefing books and scientific advice meetings – PIPs – INDs – NDAs; ODD requests), Marketing and Sales, Business Development Directorates. She is an author of approximately 70 publications.



Allen Fischer is the Regional Director of Neonatology for Kaiser Permanente in Northern California. Dr. Fischer is a graduate of the University of Pennsylvania (BA) and Stanford University Medical School. He trained in Pediatrics at Stanford, remaining for a fourth year as chief pediatric resident. He then completed a fellowship in Neonatal-Perinatal Medicine at Stanford. Dr. Fischer oversees utilization and quality improvement activities related to neonatal care for the 15 hospital Kaiser Permanente network throughout Northern California. His current areas of focus are improving the care of newborns with asymptomatic hypoglycemia and implementation of guidelines and information technology tools to reduce the use of antibiotics in term and preterm infants. He also consults for Kaiser Permanente in the Mid Atlantic States addressing issues pertaining to quality and utilization of neonatal and perinatal care. He is a member of the American Academy of Pediatrics and the California Children's Services technical advisory committee. When Allen is away from work, he enjoys riding his road bicycle throughout the Bay Area. He is also an active wine collector.

Anna Maria Hibbs is a neonatologist with expertise in neonatal epidemiology and clinical trials. She currently serves as the Interim Vice-Chair for Research for Rainbow Babies and Children's Hospital and holds the Eliza Henry Barnes Chair in Neonatology. Her research focus has been on the post-NICU pulmonary sequelae of prematurity, particularly prematurity-associated wheezing, in infants with and without a diagnosis of bronchopulmonary dysplasia. In addition, she has a research interest in the methodologic issues unique to studies of infants and NICU patients. Dr. Hibbs has authored numerous articles and also co-edited a book about the pulmonary outcomes of preterm infants.

Rosemary Higgins, a board certified neonatologist, serves as the Program Scientist for the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) Neonatal Research Network at the National Institutes of Health (NIH). In addition, she administers a portfolio of research and training grants concerned with disorders of the newborn, newborn intensive care treatments and technologies and neonatal adaptation and well-being. Dr. Higgins received her MD degree from Georgetown University, served a Pediatric Residency at the University of Virginia Medical Center, and a Neonatal-Perinatal Medicine Fellowship at the University of Rochester. She joined the faculty in 1991 at New York University Medical Center and Bellevue Hospital Center. In 1996 she moved to Georgetown University where she was the Neonatal-Perinatal Medicine Fellowship Director. In 2002, she joined the NIH at NICHD and is responsible for day to day activities in the Neonatal Research Network.



Erik Jensen is an academic neonatologist at the Children's Hospital of Philadelphia and the University of Pennsylvania. His primary areas of research focus are: 1) application of clinical and translational research methods to identify novel targets for prevention and treatment of bronchopulmonary dysplasia (BPD); 2) assessment of diagnostic test accuracy and the prognostic reliability of neonatal cardiorespiratory disease definitions; and 3) use of health services research techniques to investigate the relative contribution of hospital based factors in the development of prognostically important neonatal morbidities.

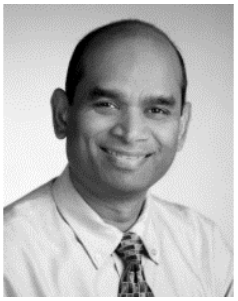
Nancy Kline Leidy is Evidera's Senior Vice President of Scientific Affairs and Senior Vice President of Patient-Centered Outcomes Research. Dr. Leidy has over 25 years of experience in clinical and health outcomes research, specializing in instrument selection, development and testing, and the design and analysis of clinical trials involving clinical, patient-reported, and functional outcomes assessment. Prior to joining Evidera, she served on the faculties of Michigan State University and the University of Arizona and as an intramural scientist at the National Institutes of Health (NIH). Dr. Leidy has presented her work at local, national, and international conferences and has over 140 papers published in refereed journals, books, and monographs. In addition to many other research activities, she is director and principal investigator of the EXACT-PRO Initiative (EXAcerbations of Chronic pulmonary disease Tool), the first patient-reported outcome (PRO) instrument development consortia. This multi-sponsor initiative brought together experts in instrument development, clinical research, and regulatory issues to develop and validate the EXACT and a derivative measure, the EXACT-RS (E-RS), for measuring outcomes in chronic obstructive pulmonary disease (COPD) trials. These PRO instruments were the first to undergo qualification review by the U.S. Food and Drug Administration (FDA), with EXACT the first PRO instrument qualified for use in drug development programs (January 10, 2014). The E-RS was the second (March 8, 2016). Both measures have also been reviewed under the European Medicines Agency (EMA) qualification of novel methodologies. Dr. Leidy holds a PhD from the University of Michigan, an MS from the University of Washington, and a BS from Michigan State University.

Alexandra Mangili, a Senior Medical Director and Global Development Lead for Shire, supports the on-going clinical needs of the Complications of Prematurity program and is primarily working on rhIGF1/IGFBP3, the company's cornerstone and high priority asset, for the prevention of morbidities related to preterm birth. Prior to Shire, she was Clinical Research Physician at Novartis/GSK Vaccines contributing to the clinical strategy of the Maternal Vaccination Program for the prevention of neonatal Group B Streptococcus sepsis and Medical Director for EMD Serono, where she designed and implemented two challenging post marketing studies for a growth hormone releasing factor in HIV Supportive Care. Alexandra received her B.S. degree from UC Berkeley and her M.D. and M.P.H. from Tufts University School of Medicine in the US. She then completed her training in Infectious Diseases at Tufts Medical Center in Boston, before joining as a staff physician and Assistant Professor of Medicine in the Division of Geographic Medicine and Infectious Diseases, where she conducted NIH-, industry- and Foundation-funded clinical research until joining industry in 2011. She practiced clinically until moving to Switzerland in 2017.

Susan McCune is the Director in the Office of Pediatric Therapeutics (OPT) in the Office of the Commissioner at the Food and Drug Administration (FDA). She joined the Agency in 2003 in the Division of Pediatric Drug Development, Office of Counter-Terrorism and Pediatric Drug Development, in the Center for Drug Development and Research (CDER). She was the Deputy Director in the Office of Translational Sciences in CDER from February, 2010, until January, 2017, when she joined OPT. Dr. McCune received her medical degree from George Washington University following her undergraduate degree at Harvard University. She completed her internship, residency, chief residency, and neonatal fellowship at Children's National Medical Center in Washington, D.C. She is Board Certified in Pediatrics and Neonatal/Perinatal Medicine. For 15 years, while practicing academic pediatric and neonatal medicine at Johns Hopkins and Children's National Medical Center, Dr. McCune continued her molecular biology research on adrenergic receptor ontogeny and expression in models of newborn brain injury in the Lab of Developmental Neurobiology, NICHD, NIH. In addition, she has a Masters in Education Technology Leadership from George Washington University, and certificates in Public Health from Georgetown and Regulatory Science from USC.

Sharon McGrath-Morrow is a Professor in the Department of Pediatrics in the Division of Pediatric Pulmonary at the Johns Hopkins School of Medicine. She has a joint appointment in the Department of Environmental Health Sciences at the Johns Hopkins Bloomberg School of Public Health. McGrath-Morrow currently serves as the Pediatric Pulmonary Fellowship Program Director at Johns Hopkins and is the PI of the T32 training grant. Dr. McGrath-Morrow received her undergraduate and medical degree from University of Virginia and her MBA from the Carey Business School at Johns Hopkins. She has been a member of the ATS since 1992 and is currently Chair-elect of the Pediatric Assembly program committee. Her areas of research include, identifying mechanisms that influence the immune response of the neonate to lower respiratory tract pathogens, the role of environmental exposures and the microbiome on respiratory symptoms in children with bronchopulmonary dysplasia and disease signatures of peripheral blood mononuclear cells in children with Ataxia telangiectasia. She receives funding from the National Institute of Health and American Academy of Pediatrics Richmond Center.

Elektra Papadopoulos is the Associate Director for the Clinical Outcome Assessments (COA) Staff in the Office of New Drugs, Center for Drug Evaluation and Research, FDA. The COA Staff contributes to a culture that ensures the patient voice is integrated into drug development through COA endpoints that are meaningful to patients, valid, reliable and able to detect meaningful change. The Staff works collaboratively to provide consultation for COAs including patient-reported outcomes used across all stages of drug development, manages the COA Drug Development Tool Qualification Program to develop and qualify COAs for unmet public health needs, and provides education and outreach to internal and external stakeholders to advance the science of COA development and implementation in drug development. Elektra joined the Staff (formerly known as SEALD, Study Endpoints Team) in 2007 as a Study Endpoints reviewer before becoming Acting Team Leader in 2013 and subsequently Acting Associate Director in 2015. She has participated in the development of guidance for the regulatory qualification of COAs and has overseen the development of an initiative to publish a compendium of COAs to promote collaboration, transparency, and communication between FDA and its stakeholders. She has also contributed to the writing of numerous guidance documents. Elektra is a board certified dermatologist. She obtained her medical degree at the University of Texas Medical Branch in Galveston. She completed her dermatology residency at the University of Texas Medical Center in Houston and dermatology fellowship at the Dermatology Branch of the National Cancer Institute before serving as Assistant Professor of Medicine and Dermatology at Georgetown University Medical Center in Washington, DC. In 2001, she joined FDA as a Medical Officer, first in the Center for Biologics Evaluation and Research and subsequently in the Division of Dermatology and Dental Products in CDER.

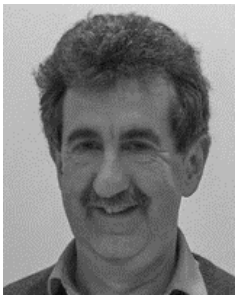


Prakesh Shah is a Professor in Department of Pediatrics and Institute of Health Policy, Management and Evaluation at the University of Toronto, Canada. He holds an Applied Research Chair from CIHR in Reproductive and Child Health Services Research. Currently, he is the director of the Canadian Neonatal Network (CNN) and an International Network for Evaluation of Outcomes of Neonates (iNeo) whereby he oversees a project of benchmarking outcomes of very low birth weight neonates in eleven countries with population-based neonatal networks with a final aim of improving quality of care across 240 NICUs participating in the network. His areas of interest include Patient and Disease oriented research in neonatal-perinatal medicine, Health services and epidemiological research in Maternal-Newborn care, Knowledge synthesis and Quality improvement.

Keira Sorrells, Founder and President of the Preemie Parent Alliance, has been leading the movement to elevate the NICU Parent Leader as a professional in maternal-infant health. After giving birth to triplets at just 25 weeks 5 days gestation and losing one of her daughters at 14 months old, Keira was propelled into maternal-infant health because of a deep desire to ensure no parent experiences the trauma of preterm birth or loss alone. Together with a dedicated team of NICU Parent Leaders, Keira has positioned the Preemie Parent Alliance to be a sought-after resource and collaborative partner for providers, researchers, industry and other stakeholders in this facet of healthcare. Parents are the key stakeholders and informants in maternal-infant health. In no other area of healthcare is it more important to have that input than in matters related to neonates; a patient population that is unable to speak for itself. Keira is passionate about strengthening the NICU Parent Leader community so that every aspect of maternal-infant health will be improved through guiding product development, research protocols, standards of

care, and educating families, communities and providers. The result of these efforts is families who thrive. Ms. Sorrells' commitment to authentic leadership and collaboration has successfully united over 30 NICU support organization leaders as a collective representation for NICU parents everywhere.

Carole Tucker has been trained as a physical therapist, electrical engineer and exercise physiologist with a primary clinical area in acute care pediatrics, human movement analysis, and the development of health outcome systems. She serves on the Functioning, Disability Reference Group of the World Health Organization (WHO). Her current research focuses on biomechanics and motor control of gait, development of patient-report outcome measures of health status in pediatric populations using modern measurement approaches, bioinformatics application in learning health systems, application of pattern recognition and advanced statistical analytical approaches to large data sets, and development and application of biosensors & related technology to improve function and mobility in individuals with disabilities. She has received funding from the Whitaker Biomedical Engineering Foundation, Shriner's Hospital for Children, NIH, and served as Co-Investigator on the NIH funded grant: Pediatric PROMIS: Advancing the Measurement and Conceptualization of Child Health. Dr Tucker is on the editorial boards of Pediatric Physical Therapy, Journal of Neuroengineering and Rehabilitation, and Physical and Occupational Therapy in Pediatrics.



Mark Turner is a Neonatologist at Liverpool Women's Hospital and the University of Liverpool. He aims to improve the access of newborn babies and children to high quality medicines. He has studied the dosing, safety and efficacy of 12 medicines in neonates with rare conditions in the past 5 years. In older children his group is currently working on manipulations of medicines, the avoidability of adverse drug reactions and the pharmacoeconomics of age-appropriate formulations. He believes that research infrastructure and data systems to optimise clinical trials are key to improving the quality of medicines. He works to develop efficient medicines research infrastructure in Europe and globally as Lead for International Liaison at the English National Institute for Health Research Clinical Research Network's Children Theme (NIHR CRN:C), Chair of the European Network for Paediatric Research at the European Medicines Agency (EnprEMA), Convenor of the European Paediatric Clinical Trials Research Infrastructure, Co-Director of the International Neonatal Consortium, Co-Scientific Coordinator of the European Commission-funded Network of Excellence, Global Research in Paediatrics (GRiP); and as Co-Coordinator of a public private partnership funded by the Innovative Medicines Initiative to set up a pan-European Paediatric Clinical Trials Network, conect4children (c4c).