Research Forum-Child Health Team Members

Team Members:

1. Irving Boime; PhD
   Professor Depts. of Developmental Biology and Obstetrics and Gynecology

2. Steven L. Brody; MD
   Professor of Medicine

3. Vikas R. Dharnidharka; MD, MPH
   Division Director, Pediatric Nephrology

4. Mary C. Dinauer; MD, PhD
   Scientific Director, Children’s Discovery Institute

5. Ann M. Doyle; RN
   Director Outpatient Research

6. Richard Head; MS
   Associate Professor of Genetics, Director of GTAC

7. Paul W. Hruz; MD, PhD
   Associate Professor of Pediatrics and Cellular Biology and Physiology

8. Sanjay Jain; MD, PhD
   Associate Professor of Medicine, Renal Division

9. Elizabeth (Betsy) Keath; PhD
   Research Forum Program Director, ICTS Navigator

10. Leslie D. McIntosh; MPH, PhD
    Assistant Professor, Pathology and Immunology

11. Kelle H. Moley; MD
    Professor and Vice Chair of Obstetrics and Gynecology

12. Margaret A. Olsen; MPH, PhD
    Associate Professor of Medicine

13. Ken B. Schechtman; MS, PhD
    Associate Professor of Biostatistics
    Associate Professor of Medicine

14. Shalini Shenoy; MD
    Professor of Pediatrics
    Medical Director, Pediatric Stem Cell Transplant

15. Mark A. Watson; MD, PhD
    Associate Professor, Pathology and Immunology

16. Neil White; MD, CDE
    Professor of Medicine and Pediatrics

17. Yi Zhang; RN, JD
    Assistant Dean for Clinical Trials
Irving Boime, PhD, Professor Depts. of Developmental Biology and Obstetrics and Gynecology

**Institution:** Washington University in St. Louis (WU)

**Expertise:** Molecular endocrinology; Structure activity relationships of pituitary peptide hormones; Protein secretion/sorting; development of biotechnology patents and multiple commercial license agreements.

**Area of Clinical or Research Interest:** Our primary research interest was the expression of gonadotropin hormone genes of the pituitary. Our work also focused on the post-translational processing of the hormones coupled to their secretion and sorting. In the course of these investigations analogs were developed that are currently being used therapeutically.

Steven L. Brody, MD, Professor of Medicine

**Institution:** Washington University in St. Louis (WU)

**Expertise:** Pulmonary, critical care, gene regulation, developmental biology, nanomaterials, epithelial cells

**Area of Clinical or Research Interest:** I have experience in both basic-translational (T1) and clinical research. My clinical research experience is primarily in critical care medicine. Our T1 research includes aspects of lung biology in chronic and acute airway disease are our major area of work. We focus on mechanisms of airway epithelial cell differentiation that characterize lung diseases such as asthma, chronic obstructive lung disease, bronchitis, bronchiectasis, post lung transplant bronchiolitis, respiratory virus and bacterial infection. We are particularly interested in mechanisms of ciliated cell differentiation and cilia biogenesis. These studies are linked to investigation of the genetic basis of lung disease in patient populations with known or suspected defects in cilia, co-called ciliopathies. Our methods utilize both animal models and human samples. Approaches involve techniques in cellular, molecular, and development biology and also employ genetic methods in human and mouse analyses. We have additional projects focused on gene therapy and related approaches, using viral vectors and non-viral nanoparticles for nucleic acid delivery and lung imaging in collaboration with colleagues in radiology.

Vikas R. Dharnidharka, MD, MPH, Division Director, Pediatric Nephrology

**Institution:** Washington University in St. Louis (WU)

**Expertise:** Pediatrics, nephrology, transplantation, epidemiology

**Area of Clinical or Research Interest:** I am a patient-oriented researcher with interests in chronic renal failure, pediatric kidney transplantation and post-transplant infections. I perform epidemiological analyses of very large national databases to elucidate risk factors for events and the outcomes after events, typically infectious events. These large databases include UNOS, USRDS, NAPRTCS. I also participate in multi-center clinical and mechanistic research trials in the areas of chronic renal insufficiency, dialysis and transplantation. My work gets funding from NIH institutes (NIDDK, NIAID) and industry. I am particularly known for my work related to a post-transplant malignancy called post-transplant lymphoproliferative disorder (PTLD), caused in most cases by Epstein-Barr virus infection. These issues of infection and malignancy post-transplant have received a lot of attention as we gave our patients more immunosuppression in the hopes of reducing acute rejection rates. My collaborators are numerous. For the prospective clinical trials, my collaborators include pediatric nephrology colleagues at various centers such as Harvard, Johns Hopkins,
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University of Pennsylvania, UCSF, University of Washington and UAB. For my epidemiological studies, I collaborate with an adult nephrologist in the Army, with biostatisticians and epidemiologists at Emmes Corporation and with a biostatistician at UNOS.

Mary C. Dinauer, MD, PhD, Professor of Pediatrics, Scientific Director, Children’s Discovery Institute

Institution: Washington University in St. Louis (WU)
Expertise: Hematology, neutrophil, inflammation, innate immunity, NADPH oxidase
Area of Clinical or Research Interest: I have been an NIH-funded investigator since 1991. My research focuses on the regulation of oxidant production by the phagocyte NADPH oxidase, its role in innate immunity and inflammation and inherited defects in Chronic Granulomatous Disease (CGD), where we recently identified a new genetic subgroup of this disorder. The NADPH oxidase is increasingly recognized to have immunomodulatory functions in addition to its role in microbial killing. My laboratory is investigating the regulation and assembly of NADPH oxidase in phagocytic leukocytes, its role in innate immunity and inflammation, inherited defects in CGD, and strategies for treatment of CGD by gene modification of hematopoietic stem cells. We have developed both a human myeloid cell line model and mouse model of X-linked CGD using gene targeting for use as pre-clinical model systems, in addition to studies in human neutrophils and marrow cells from healthy controls and patients with CGD.

Ann M. Doyle, RN CCRC, Director of Outpatient Research

Institution: Washington University in St. Louis (WU)
Expertise: Hematology, neutrophil, inflammation, innate immunity, NADPH oxidase
Area of Clinical or Research Interest: I have been coordinating clinical trials for more than 15 years; my area of was primarily Inpatient ICU trials along with outpatient pulmonary clinical trials. My experience ranges from Procedural, to budgeting, to regulatory to mentoring. In addition I currently am the director of the outpatient research facility in the CAM, providing research space, support for researchers/coordinators here at the university. We support a great deal of Phase I cancer trials rheumatology, neuromuscular, pulmonary, gastroenterology, psychiatry, solid organ transplant among other disciplines. Our goal is to provide dedicated space and personnel for researchers for industry, departmental, and NIH sponsored trials.

Richard Head, MS, Research Associate Professor of Genetics

Institution: Washington University in St. Louis (WU)
Expertise: Genomics, Inflammation & Immunology, Drug Development, Computational Biology
Area of Clinical or Research Interest: 17 years in pharmaceutical/medical research. Currently the director of the Genome Technology Access Center (GTAC) and director of R&D for Genomics and Pathology Services (GPS). My lab at Pfizer was focused on all aspects of research in inflammation &immunology from new target discovery to translational/clinical research with the use of multiple 'omics technologies and computational biology. Two of our major achievements were the determination of the
mode of action of the recently approved Xeljanz (JAK inhibitor) and the development of an extremely sophisticated and detailed molecular/cellular level model of Rheumatoid Arthritis. Since returning to Washington University two years ago, it has been my goal to apply those translational/clinical learnings more broadly through the GTAC and GPS in multiple areas of disease research. Most recently we have successfully moved multiple cutting edge assays from the research space to use in patient care and clinical trials.

Resources: GTAC, Genomics and Pathology Services

Paul W. Hruz, MD, PhD, Associate Professor of Pediatrics and Cellular Biology and Physiology

Institution: Washington University in St. Louis (WU)
Expertise: Diabetes, HIV, Protein structure/function, Drug design, Membrane transporters
Area of Clinical or Research Interest: Dr. Hruz is board certified in Pediatrics and Pediatric Endocrinology and Metabolism. Dr. Hruz has clinical interest in a wide range of endocrine disorders, with a special interest in diabetes mellitus. His research efforts are directed toward understanding facilitative glucose transport as it relates to normal and disordered glucose homeostasis. The laboratory is investigating the in vitro and in vivo effects of HIV protease inhibitors on glucose transporter function. The goal of this research is to identify the molecular mechanisms that lead HIV infected patients receiving PIs to develop insulin resistance. The laboratory is also using these isoform-selective antagonists of the insulin-responsive transporter GLUT4 to understand the role of glucose transport in insulin-responsive tissues. Recent efforts have been directed toward understanding the role of glucose transport in the failing heart. This has led to translational research into the understanding of the influence of insulin resistance in pediatric heart failure.

Sanjay Jain, MD, PhD, Associate professor of Medicine (Renal Division), Pathology & Immunology; Director-Kidney translational Research Core

Institution: Washington University in St. Louis (WU)
Expertise: molecular genetics, translational research, kidney and urological disease research
Area of Clinical or Research Interest: Our overall interest is to delineate molecular signals that regulate normal development, injury and regeneration of the genitourinary system. We use glial cell line-derived neurotrophic factor (GDNF) family ligands-Ret receptor tyrosine kinase signaling system as a paradigm. This signaling system is associated with a number of human diseases including developmental abnormalities, neurodegenerative diseases and cancer syndromes. Using genetically defined Ret signaling mutants we have identified molecular basis for signaling specificity in the development of urogenital system and autonomic nervous system. A major area of interest is renal and urinary tract malformations and gene-environment interactions between the GU and the peripheral nervous system. To this end we are using innovative mouse models, genomic approaches in exome sequencing of patients with these disorders and genome wide expression studies to understand fundamental mechanisms, causative genes and developmental programs necessary for GU and nervous systems to work in harmony in normal and disease states.
Elizabeth (Betsy) Keath PhD, Research Forum Program Director, ICTS Project Manager and Navigator for Translational Research

Institution: Washington University in St. Louis (WU)
Expertise: Molecular medical mycology, molecular/cellular immunobiology in host response, mentoring.
Area of Clinical or Research Interest: My interests include the development and implementation of efficient, structured processes and guidance to improve and accelerate project and biotechnology development activities for clinical and translational research. I currently serve as Senior Project Manager and Navigator for Translational Research for the ICTS with a trio of responsibilities: 1) Project management as program officer for the BJHF/ICTS Pilot Award program and the Just-In-Time Core Usage program; 2) Liaison to the ICTS partner institution; and 3) Research resource navigation to cores and services for ICTS members.
Resources: ICTS, eNavigator tool

Leslie D. McIntosh, BA, MPH, PhD; Research Assistant Professor, Pathology and Immunology

Institution: Washington University in St. Louis (WU)
Area of Clinical or Research Interest: My interests include using technology to translate and disseminate health data; for example, developing on-line and computer aided surveys in order to reduce input errors, decreasing the time to collect survey data, and increasing user accessibility of the survey. By accessibility I mean modifying a survey (e.g. enlarging the font size, offering voice-interactive modes) to make it more user-friendly. Additionally, the lifecycle of surveys - the development, testing, deployment, and analyses of the instruments and data - is of interest in my research endeavors. I would also like to develop better ways to improve the presentation of data to end-users. I am always interested in learning new techniques to analyze data, such as conjoint analysis and social network analysis.
Resources: ICTS CBMI core and informatics

Kelle H. Moley, MD, James P. Crane Professor and Vice Chair of Obstetrics and Gynecology, Professor, Vice Chair of Basic Science Research, Director, Basic Research Division

Institution: Washington University in St. Louis (WU)
Expertise: Reproduction, development, biorepository, cell biology
Area of Clinical or Research Interest: Dr. Moley is a Reproductive Endocrinologist and Professor of Obstetrics and Gynecology. She is currently Vice Chair and Director of the Division of Basic Science in Women’s Reproductive Science Research. She is also the Co-Director of the Washington University Institute for Clinical and Translational Science. She is PI and Director of the Reproductive Scientist Development Program, a national K12 training program now in its 26th year. She is Program Director of the Women’s Reproductive Health Research Center and PI of a T32 in Reproductive Sciences at Washington University School of Medicine. Dr. Moley also co-founded the Women and Infant’s Health Specimen Consortium (WIHSC) at Washington University in St. Louis, the largest longitudinal, pregnancy tissue biorepository in North America. Dr. Moley’s research has impacted our understanding of reproductive performance and glucose utilization in diabetic and obese animal models and how these findings are applicable to the pathophysiology of diabetes in humans. Recently her work has focused on developmental
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origins of adult disease, specifically the susceptibility of offspring to adult diseases such as metabolic syndrome and cancer.

Resources: WIHSC repository

Margaret A. Olsen, BS, MPH, PhD, Research Associate Professor of Medicine and Surgery
Email, Website

Institution: Washington University in St. Louis (WU)
Expertise: Epidemiology, infectious diseases, administrative data, observational data
Area of Clinical or Research Interest: My primary area of research concerns the epidemiology of healthcare associated infections, including the incidence of infection after a variety of different surgeries, risk factors and outcomes of infection. I am also interested in developing computerized methods of surveillance for surgical site infections and other healthcare associated infections, using administrative and electronic data. I am using large administrative databases to perform surveillance for healthcare associated infections and for comparative effectiveness research. I am very interested in the use of these large generalizable datasets to compare outcomes after medical and surgical treatments, including the role of facility-level characteristics.

Resources: CADR core

Ken B. Schechtman, BS, MS, PhD, Associate Professor of Biostatistics, Research Associate Professor of Medicine
Email, Website

Institution: Washington University in St. Louis (WU)
Expertise: clinical trials design, statistical analysis of data, multicenter clinical trials, epidemiologic methods
Area of Clinical or Research Interest: My primary areas of interest involve clinical trials research methodology, the conduct of clinical trials, and the statistical evaluation of clinical trials data. Major areas of application include cardiology, obesity in children and adults, exercise physiology, and asthma. My central concerns in these clinical and translational research projects include developing and implementing study designs that minimize bias and enhance the likelihood that the planned study will confidently address the research questions of interest and ensuring that research projects are conducted using procedures that further the goals of the study. Additional areas of interest include the design and conduct of epidemiological research.

Resources: Research Design & Biostatistics Group (RDBG)

Shalini Shenoy, MD, Teresa J. Vietti MD Scholar in Pediatrics, Professor of Pediatrics, Medical Director, Pediatric Stem Cell Transplant Program
Email, Website

Institution: Washington University School of Medicine, St. Louis Children’s Hospital
Expertise: patient oriented research, hematology and oncology, bone marrow transplant
Area of Clinical or Research Interest: My academic focus is on the development of safer less toxic methods of hematopoietic stem cell transplantation in children. Toward this, I am investigating reduced intensity transplantation for children with
hemoglobinopathy (sickle cell disease and thalassemia) using the best available related or alternate donors.

**Mark A. Watson, BA, MD, PhD, Associate Professor, Pathology and Immunology**

**Institution:** Washington University in St. Louis (WU)

**Expertise:** Genomics, Cancer, Biomarkers

**Area of Clinical or Research Interest:** Application of whole genomic technologies (e.g. DNA microarrays) and biomedical informatics approaches to the molecular characterization of breast cancer and other malignancies. Development and validation of genome-based biomarkers for the prognosis and targeted therapeutic management of cancer.

**Resources:** ICTS Translational Pathology & Biomarker Development resource

**Neil H. White, MD, CDE, Professor of Pediatric and Medicine, Director, Pediatric Clinic Research Unit**

**Institution:** Washington University in St. Louis (WU)

**Expertise:** Pediatrics diabetes, endocrinology, hypoglycemia

**Area of Clinical or Research Interest:** As the Director of the Pediatric Clinical Research Unit (PCRU) for Washington University in St. Louis I am involved in numerous childhood disease processes. My primary discipline is endocrinology, is involved on both the national and international level for the research and treatment of diabetes. I am involved in multiple facets of diabetes from prevention to treatment of the disease. My expertise also includes Wolfram Syndrome a rare genetic variation of diabetes. I am also supported by the Clinical and Translational Science Award grant, and various grants from pharmaceutical companies. I am also a principal investigator for institutionally-based fellowship training (T32) and career-development (K12) grants from NIDDK.

**Resources:** Pediatric Clinical Research Unit

**Yi Zhang, JD, RN, Assistant Dean Clinical Trials**

**Institution:** Washington University in St. Louis (WU)

**Expertise:** Clinical research management; operational & financial expertise in managing research units and research coordinators; FDA (GCP) and Common Rule related regulatory experience; Contracts/budgets negotiations with industry.

**Area of Clinical or Research Interest:** Interested in understanding and providing the best operational infrastructure to support clinical and translational research. Have experience in how to approach industry for clinical research funding and negotiate contracts/budgets. Knowledgeable in FDA and OHRP regulatory requirements for human subject research.

**Resources:** ICTS CARS (Clinical Research Units), Contracts, Regulatory, Study Coordinator and Recruitment oversight