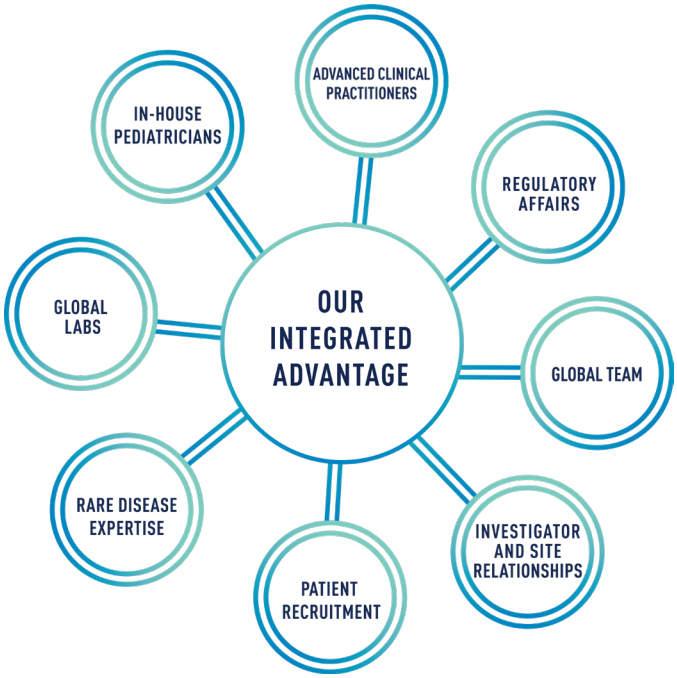
MEDPACE

A DEEPER DIVE INTO PEDIATRIC CLINICAL RESEARCH

Pediatric clinical research presents a unique set of challenges and considerations. To design and conduct these studies, you need a team of highly qualified and experienced medical, regulatory, and operational experts who understand and can navigate the many complexities.

Pediatric subjects are specialized given their unique and fundamental differences from adults. These include physiological and biological differences, the dynamic changes due to growth and development, their attitudes, perceptions of the world, and their psychological outlooks. Pediatric trials involve different requirements for consent making it more complex. The vulnerability of children demands special expertise with regard to pediatric investigators and awareness of the intricate ethical issues associated with conducting these trials. Medpace understands the unique concerns raised by ethics committees, investigators, and parents, and can help develop strategies to address specialized needs.



DEEP AND BROAD PEDIATRIC EXPERIENCE AND EXPERTISE

- Medpace has built an experience base in the management of pediatric trials throughout our 30 year history
- In-house pediatricians and board-certified advanced clinical practitioners provide invaluable insights for designing and conducting trials
- Dedicated global Regulatory Affairs helps develop strategy and navigate the requirements for pediatric trials
- Our global operations support pediatric recruitment and execution of studies around the world while maintaining ethical considerations at both local and global levels
- Close working relationships with investigators and site networks support more accurate feasibility, better recruitment and improved retention
- Extensive experience conducting studies in rare disease and orphan indications provides additional expertise that drives efficiency
- Global laboratory services including central laboratory, bioanalytical, ECG core lab, and imaging core labs provide increased efficiency for addressing the testing components of pediatric trials

EXPERTS

PEDIATRIC EXPERTS FOR SCIENTIFICALLY-DRIVEN STUDIES

Our team of in-house pediatricians have extensive clinical experience treating sick children as well as designing, conducting, and managing pediatric trials. Medpace's scientifically-driven approach to clinical research gives Sponsors the advantage of early and ongoing insight and guidance from these therapeutic experts throughout the trial design and execution. Our medical doctors provide strategic direction for study design and planning, train operational staff, work with Investigators, provide medical monitoring, and meet with regulatory agencies. They are embedded throughout your studies, providing greater depth and the ability to tackle complex and challenging diseases, with a deep understanding of pediatric populations. They help navigate ethics committee reviews by working closely with sites to lower the risk of lengthy negotiations, deficiencies and/or queries. Based on their deep understanding of the population and disease, our pediatricians help ensure that the study design is patient- and parent-centric to increase participation and ultimately, project success.

Franklin O. Smith, III, MD, FAAP, FACP
Sr. Vice President, Medical Department

Blythe Thomson, MD
Sr. Vice President, Medical Department

Brian Murphy, MD, MPH
Vice President, Medical Department

Ann Woolfrey, MD
Vice President, Medical Department

Mariko DeWire-Schottmiller, MD
Sr. Medical Director

Gregory Hale, MD, FAACP, MBA
Sr. Medical Director

Yulia Lurye, MD
Sr. Medical Director

Ineta Sosare, MD, PED, NEP
Sr. Medical Director

Xiuhua "Liang" Bozarth, MD, PhD
Medical Director

Arlene Dent, MD, PhD
Medical Director

Adam Lubert, MD
Medical Director

PEDIATRIC AND ADVANCED CLINICAL PRACTITIONERS ADD A PATIENT'S PERSPECTIVE

Our medical directors are supported by a team of experienced ACPs whose unique perspective into pediatric patients brings added value to the clinical development team. These highly-trained, board-certified experts provide an additional layer of knowledge and insights that can streamline your studies and address potential challenges early in the study planning.

In pediatric studies, it is especially important to carefully consider study procedures to ensure they are tailored to children and their parents or guardians — the ACPs add crucial insights to ensure your study can be operationalized. Like our medical doctors, ACPs are embedded in the teams to ensure your study is feasible and operationalized for efficient execution by looking at the protocol through the lens of the patient, the parents, and site staff. ACPs also help with the review of safety events, ongoing team education, recruitment strategies, and study document review. Their understanding of what works in a clinical setting makes them a valuable part of our embedded medical expertise model.

Laura Clark, APRN
Advanced Clinical Practitioner

IN-HOUSE PEDIATRICIAN BIOGRAPHIES

Franklin O. Smith, III, MD, FAAP, FACP

Sr. Vice President, Medical Department, Hematology and Oncology

Dr. Frank Smith is a clinical hematologist/oncologist with over 30 years of experience as a Principal Investigator on clinical trials.

Experience Summary

- Three decades of academic clinical practice and research in pediatric hematology/oncology and adult hematopoietic cell transplantation
- Principal Investigator or co-investigator on numerous Phase I, II, and III clinical and translational research studies in acute myeloid leukemia, fanconi anemia, cord blood transplantation and gene therapy
- 180 peer-reviewed scientific manuscripts, reviews, chapters and books
- Professor of Medicine and Pediatrics, University of Cincinnati College of Medicine
- Previously: Clinical Director, University of Cincinnati Cancer Institute; Director, Division of Hematology/Oncology, Cincinnati Children's Hospital Medical Center; Vice-Chair, Children's Oncology group

Education Summary

- Doctor of Medicine, University of South Carolina School of Medicine
- Residency in Pediatrics, University of Florida College of Medicine
- Fellowship, Pediatric Hematology/Oncology, University of Washington and the Fred Hutchinson Cancer Research Center

Blythe Thomson, MD

Sr. Vice President, Medical Department, Hematology and Oncology

Dr. Blythe Thomson is board certified in pediatric hematology and oncology and brings over 25 years of clinical trial and drug development experience to the Medpace team.

Experience Summary

- Over 25 years of experience in medical management of patients with solid tumors and hematologic malignancies, including cell based therapy and hematopoietic cell transplant
- Experienced managing outpatient and inpatient oncology centers, apheresis units, as well as clinical and translational research units
- Drug development experience in all phases of clinical development for treatment of solid tumors, benign and malignant hematology
- Well versed in global regulatory submissions and pediatric investigational plans

Education Summary

- Doctor of Medicine, The Ohio State University
- Residency in Pediatrics, The Ohio State University
- Fellowship, Pediatric Hematology/Oncology, Fred Hutchinson Cancer Research Center
- Certificate Program, Healthcare Administration, University of Washington
- Masters of Business Administration, University of Massachusetts

Brian Murphy, MD, MPH

Vice President, Medical Department, Infectious Diseases and Vaccines

Dr. Brian Murphy is board certified in infectious diseases, internal medicine, and pediatrics and has over 12 years of experience in clinical research for anti-infectives and vaccines.

Experience Summary

- Extensive industry experience in Phase I-IV global infectious disease studies, including antibacterials, antifungals, antivirals, vaccines, and device
- Eight years' experience as an academic PI and consultant on multiple infectious disease programs investigating the pathogenesis and management of serious infections in hospitalized patients, HIV, pulmonary respiratory pathogens, fungal diseases, vaccine development, bioterrorism, and immunotherapeutics
- Leadership roles in multiple translational and clinical research projects sponsored by the NIH (NIAID and NHLBI), CDC, Department of Defense, US Department of Health and Human Services, and other non-dilutive funded-projects in a broad range of patient populations
- Upholds an academic appointment and clinical practice and maintains close ties with his clinical and research colleagues who participate in clinical and translational research

Education Summary

- Doctor of Medicine, University of Louisville, School of Medicine Residency, University of Kentucky, School of Medicine
- Fellowship, Infectious Disease, University of Kentucky, School of Medicine
- Master of Public Health, University of Kentucky, College of Public Health
- Fellowship, Vaccine Research, NIH Center of Excellence for Bioterrorism

Anne Woolfrey, MD

Vice President, Medical Department, Hematology and Oncology

Dr. Ann Woolfrey is board-certified in pediatric hematology-oncology and internal medicine. She brings 27 years' experience in academic clinical research in the fields of hematology/oncology and cell therapies.

Experience Summary

- Faculty member at a leading cancer research center and at a recognized academic institution for over 20 years, rising to Full Member and Professor ranks, respectively
- Active member in four professional affiliations revolving around pediatrics, hematology, bone marrow transplant, and histocompatibility and immunogenetics
- Board Certified in internal medicine as well as pediatric hematology and oncology
- Key opinion leader with over 150 publications and presentations in the fields of hematology, oncology, transplant immunology, and pediatrics
- Experience in clinical research for over 30 years

Education Summary

- Bachelor of Arts in Chemistry, Saint Olaf College
- Doctor of Medicine, University of Minnesota
- Residency in Pediatrics and Internal Medicine, University of Chicago Hospitals and Clinics
- Fellowship, Pediatric Hematology/Oncology, Children's Hospital and Medical Center/Fred Hutchinson Cancer Research Center

Mariko DeWire-Schottmiller, MD

Sr. Medical Director, Hematology and Oncology

Dr. Mariko DeWire-Schottmiller is a board-certified pediatric hematologist/oncologist with 15 years of experience in clinical research, academia, and clinical oncology practice in Oncology and Neuro-oncology.

Experience Summary

- Over 15 years of experience in medical management of patients with malignancies including brain tumors
- Previously, Associate Professor, at University of Cincinnati, College of Medicine, Cancer and Blood Diseases Institute, Cincinnati Children's Hospital Medical Center
- Investigator experience in trials involving neuro-oncology, malignant brain tumors, pediatric brain tumors, and solid tumors
- Leadership roles in The International DIPG/DMG Registry, Pediatric Brain Tumor Consortium, and Children's Oncology Group
- Active member of ASCO, SNO, AACR, and ASPHO
- Well published in peer-reviewed medical and scientific literature
- Peer reviewer for multiple oncology journals

Education Summary

- M.D., Boonshoft School of Medicine at Wright State University
- Residency in Pediatrics, University of Tennessee College of Medicine
- Fellowship, Pediatric Hematology/Oncology, University of Tennessee College of Medicine and St. Jude Children's Research Hospital
- Neuro-Oncology training, Department of Oncology, Neuro-Oncology Division, St. Jude Children's Research Hospital

Gregory Hale, MD, FAAP, MBA

Sr. Medical Director, Hematology and Oncology

Dr. Gregory Hale is a board-certified pediatric hematologist- oncologist with over 24 years of clinical trial experience in hematopoietic cell transplantation, cellular and gene therapies, immunodeficiencies, non-malignant hematology, hematologic malignancies and solid tumors.

Experience Summary

- Held a variety of director level roles at nationally recognized academic institutions where he served as a principal investigator or co-investigator for pilot and Phase I-III clinical trials
- Investigator experience in trials involving benign hematology, vaccines, immunotherapy, chemotherapy, cellular and gene therapies, hematopoietic cell transplantation
- Leadership roles in Center for International Blood and Marrow Transplant Research (CIBMTR), National Marrow Donor Program (NMDP), Foundation for the Accreditation of Cellular Therapy (FACT), and American Academy of Pediatrics (AAP)
- Thought leader with >210 peer-reviewed manuscripts, book chapters, & review articles
- Previously Professor of Oncology and Pediatrics at Johns Hopkins University School of Medicine and Medical Director, Pediatric Hematology Oncology, Johns Hopkins All Children's Hospital
- Previously Clinical Director, Division of Blood and Marrow Transplantation, and Medical Director, Transplant and Gene Therapy Clinical Research Office, St. Jude Children's Research Hospital
- Active member of ASH, ASCO, ASBMT, AAP, ISCT
- Medical Monitor experience in Phase 1, 2, and 3 clinical trials, including first in human and global trials

Education Summary

- Doctor of Medicine, Summa Cum Laude, Joan C. Edwards School of Medicine at Marshall University.
- Residency in Pediatrics, Children's Hospital of Pittsburgh of UPMC
- Fellowship, Pediatric Hematology/Oncology, St. Jude Children's Research Hospitals

Yulia Luryve, MD

Sr. Medical Director, Nephrology and Hepatology

Dr. Yulia Lurye is a clinical research physician with board-certifications in endocrinology and pediatric endocrinology and nephrology.

Experience Summary

- 10+ years of clinical trial experience, including seven years as an investigator and seven as a medical director working across all phases within a Clinical Research Organization
- Therapeutic areas include metabolic disorders, cardio-vascular, infectious diseases, kidney/ renal and liver diseases, transplantation & immunosuppressive therapy, rare diseases, and growth disorders. Therapeutic modalities/products include advanced gene and cell therapy, biologics and biosilimilars, medical devices
- 10+ years of clinical practice in diabetes and CKD, & years of clinical practice in transplantation of solid organs (kidney, pancreas, liver, intestine, heart, lung) into adults and children
- Research work on predictive models of survival after solid organ transplantation, bone marrow stem cell therapy and IGF-1/growth hormone imbalances in children with liver cirrhosis

Education Summary

- Doctor of Medicine, Russian State Medical Academy (Honors)
- Residency in internal medicine
- Fellowship in endocrinology, pediatric endocrinology and nephrology
- Holds certificates in endocrinology, pediatric endocrinology and nephrology, liver transplantation, kidney and pancreas transplantation.

Inetea Sosare, MD, PED, NEP

Sr. Medical Director, Nephrology

Dr. Ineta Sosare is a board-certified nephrologist and pediatrician with more than 18 years of therapeutic expertise gleaned from her roles as a physician and in academia.

Experience Summary

- Extensive medical monitor experience in phase II-III global studies in autoimmune disorders, glomerulonephritis, chronic kidney disease, kidney transplant, and pediatric trials including phenylketonuria, familial hypercholesterolemia, infantile hemangioma
- Experience as a consulting nephrologist and medical faculty lecturer for pediatrics and nephrology
- Provided medical expertise to project teams on clinical drug development, throughout the lifecycle of compounds, including supporting study design and generating study protocol
- Provided safety and protocol training to sites and study teams

Education Summary

- Diploma in Nephrology, Medical Academy of Latvia – Riga, Latvia
- Diploma in Paediatrics, Medical Academy of Latvia – Riga, Latvia
- Doctor of Medicine, Medical Academy of Latvia – Riga, Latvia

Xiuhua "Liang" Bozarth, MD, PhD

Medical Director, Neuroscience

Dr. Bozarth is a board-certified neurologist with special qualifications in child neurology and epilepsy.

Experience Summary

- 13+ years of clinical experience in academic medicine and clinical research
- Prior academic appointment and Principal Investigator (PI) work performed at Seattle Children's Hospital and University of Washington
- Served as a PI for multiple clinical trials, observational studies, and investigator-initiated drug studies
- In-depth knowledge in early onset catastrophic intractable epilepsies
- Strong expertise in pediatric rare genetic disorders from clinical diagnosis/management, basic scientific research, and clinical trials
- Therapeutic expertise in child and adult neurology, including epilepsy, neurogenetic disorders, neuromuscular diseases, and neurodevelopmental disorders
- Multiple publications of original research in peer-reviewed journals

Education Summary

- Doctor of Philosophy in Neuroscience, The University of Hong Kong – Hong Kong, Hong Kong
- Bachelor of Medicine, Bachelor of Surgery, China Medical University – Liaoning, China

Arlene Dent, MD, PhD

Medical Director, Infectious Diseases and Vaccines

Dr. Arlene Dent is board-certified in Pediatrics and Pediatric Infectious Diseases with over 20 years of clinical experience in inpatient and outpatient pediatric Infectious Diseases practice.

Experience Summary

- Prior NIH funded research focused on human immunologic responses to infectious diseases using state-of-the-art technology
- Experience includes working with premature severely ill babies in the Neonatal Intensive Care Unit, patients in the Pediatric Intensive Care Unit, Transplant and Hematologic/Oncologic patients who may be severely immunosuppressed, and general pediatric patients admitted with severe infections
- Experience developing research plans, including grant applications, grant project implementation, participant safety monitoring, research study procedures, and data analysis
- Well-published in peer-reviewed articles and publications on pediatric infectious diseases

Education Summary

- Doctor of Philosophy in Anatomy, Indiana University - Bloomington, IN
- Doctor of Medicine, Indiana University - Bloomington, IN
- Bachelor of Science in Biology, Indiana University - Bloomington, IN

Adam Lubert, MD

Medical Director, Cardiovascular

Dr. Adam Lubert is a pediatric and adult congenital cardiologist with over 10 years of experience in clinical practice, academia, and clinical research.

Experience Summary

- Board-certified in internal medicine, pediatrics, pediatric cardiology, and adult congenital heart disease
- Served as a pediatric and adult congenital cardiologist on faculty at Cincinnati Children's Hospital
- Directed the Fontan Management Program and led a multidisciplinary team of healthcare providers to optimize the care of children and adults who underwent the Fontan procedure
- Key opinion leader, authoring over 40 peer-reviewed articles in the fields of pediatric and adult congenital cardiology

Education Summary

- Doctor of Medicine, Wayne State University – Detroit, MI
- Bachelor of Science in Chemistry, University of Michigan – Ann Arbor, MI

Laura Clark, MSN, APRN, FNP-BC

Advanced Clinical Practitioner, Hematology and Oncology

Laura Clark is an advanced clinical practitioner specialized in the medical management, health promotion, and health maintenance of adults with hematologic and oncologic diseases.

Experience Summary

- Specialized in the medical management, health promotion and health maintenance of children, adolescents, and young adult patients with leukemia/lymphoma, immunodeficiency, and bone marrow failure patients pre- and post-HCT in the outpatient and day hospital setting
- Served on the quality review council in the Cancer and Blood Diseases Institute
- Provided education to the patients and families regarding disease process, treatment, medications, and related side effects and outcomes
- Specialized in the medical management of children, adolescents, and young adult patients pre- and post-operatively in the hospital
- Provided care for routine and acute visits, blood transfusions, infusion therapy, bone marrow procedures, and chemotherapy treatments
- Performed bone marrow biopsies and aspirates on adult patients in the outpatient setting
- Provided direct oversight of the hematology/oncology patients receiving chemotherapy treatments, transfusions, and infusions

Education Summary

- Master of Science in Nursing, Georgia College and State University
- Bachelor of Science in Nursing, Old Dominion University

EXECUTION

REGULATORY EXPERTISE

Globally, clinical research in pediatrics is growing at a rapid rate based on regulatory and legislative demands for initiation of safety and efficacy standards during drug development. Sponsors are encouraged to develop new therapeutics in response to governmental incentives regarding pediatric exclusivity for compounds in development. This focus on pediatrics brings a new level of regulatory complexity. The FDA Reauthorization Act (FDARA) and the RACE for Children Act are two examples of the increased pressure to accelerate drug development for pediatric cancer patients.

Medpace's Regulatory Affairs helps Sponsors formulate pediatric development strategies (Pediatric Study Plans (iPSP) and Pediatric Investigation Plans (PIP)) as well as coordinate each aspect of engagement — balancing strategy, data, analysis, and timelines by globally optimizing regulatory interactions and strategic approaches.

Additionally, our start-up teams understand country-specific and regional regulations, enabling us to develop proactive solutions to regulatory issues and challenges within achievable timelines. Our team of dedicated specialists coordinate and oversee study submissions to Regulatory Authorities and Ethics Committees, facilitate essential document collection, and review and finalize contract and payment documentation at project start-up and throughout the study. We rigorously maintain timelines for study start-up, ensuring your trial is initiated on-time and on-budget.

GLOBAL TEAMS

With coverage across six continents, Medpace has the global expertise and experience to proactively plan and execute trials of all sizes. Our medical, operational, and regulatory specialists have country-specific expertise which allows them to acquire a deeper understanding of the importance of local language, culture, and processes. By being embedded in the culture, we can create better relationships with investigators, deliver faster enrollment timelines, and obtain access to the country-specific pediatric patient populations.

INVESTIGATOR AND SITE RELATIONSHIPS

Pediatric sites must be carefully selected or a study can quickly stall. To best prepare for and mitigate potential delays, Medpace leverages our relationships with investigators for more accurate feasibility, better recruitment and improved retention allowing for Sponsors to meet project milestones in these challenging studies. We work closely with sites to lower the risk of lengthy negotiations, deficiencies and/or queries. Additionally, our experience working with academic groups and pediatric research networks is a fundamental contributor to successful enrollment and start-up. Knowing that regulatory timelines at some academic institutions are often substantially longer than those that use centralized IRBs, the Medpace study team works to deepen relationships with the sites as soon as we begin work.

PATIENT RECRUITMENT AND RETENTION

Trials involving children and adolescents are particularly challenging for recruitment and retention including unique emotional issues for children with disease, social/family dynamics, as well as practical issues such as scheduling, transportation, drug administration, storage, and compliance. Our dedicated patient recruitment and retention team works hand-in-hand with the project team and employs creative strategies to reach recruitment and retention goals.

RARE DISEASE EXPERTISE

Many pediatric studies are focused on rare disease and orphan indications, adding yet another layer of complexity. There are additional regulatory considerations, patient recruitment hurdles, and significant study design implications. Medpace applies its extensive experience in rare disease clinical development to provide the insights, guidance, and relationships to conduct these studies. A significant advantage is the combined scientific input from our in-house pediatricians, as well as MDs specialized in the targeted therapeutic area, and advanced clinical practitioners who bring practical insights from the clinician and patient, and family perspectives. Our Regulatory Affairs team is well-versed in the requirements for both pediatrics and rare diseases and our Patient Recruitment and Retention team works directly with advocacy groups to optimize study participation.

GLOBAL LABS

Through its wholly-owned subsidiaries, Medpace offers supporting laboratory services including central laboratory, bioanalytical lab, ECG core lab, and imaging core labs. This integrated approach can streamline study execution and minimize vendor oversight. Our scientific and operational experts from each of these service areas work hand-in-hand with the core project team. For pediatric trials, this ensures that the special needs for testing are considered and incorporated into the trial design.

FULL-SERVICE CLINICAL DEVELOPMENT

Medpace is a scientifically-driven, global, full-service clinical contract research organization providing Phase I-IV clinical development services to the biotechnology, pharmaceutical and medical device industries. Medpace's mission is to accelerate the global development of safe and effective medical therapeutics through its high-science and disciplined operating approach that leverages local regulatory and deep therapeutic expertise across all major areas including oncology, cardiology, metabolic disease, endocrinology, central nervous system and anti-viral and anti-infective.

WE CAN'T SIMPLIFY CLINICAL DEVELOPMENT – BUT WE CAN EXECUTE IT SEAMLESSLY.

MAKING THE COMPLEX SEAMLESS®