

# 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.

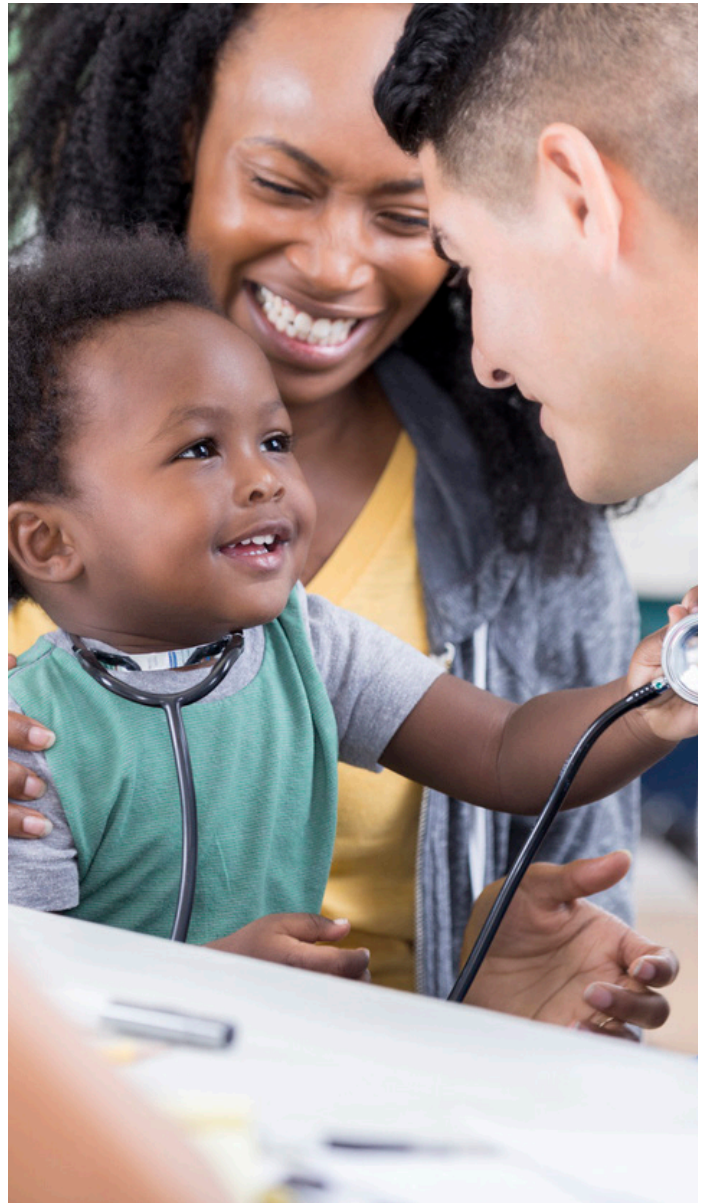
## DEEP AND BROAD PEDIATRIC EXPERIENCE AND EXPERTISE

- 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



## 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*



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



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



**Adam Lubert, MD**  
*Medical Director*



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## 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



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## **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

## **Ann Woolfrey, MD**

*Vice President, Medical Department,  
Hematology and Oncology*

Dr. Ann Woolfrey is board-certified in pediatric hematology-oncology and internal medicine. She brings over 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



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## 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

## Ineta 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



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## 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



## 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.



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## 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.**

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MAKING THE COMPLEX  
**SEAMLESS**<sup>®</sup>

