Conducting Pediatric Studies Presents Great Challenges.

Look to a CRO with noted pediatric physicians across multiple therapeutic areas

Clinical development in a pediatric population demands that the CRO has both a thorough medical understanding of the disease, as well as experience working with this patient population. Pediatric trials involve unique challenges including recruiting, inclusion/exclusion criteria, developmental aspects of drug metabolism as well as subject compliance and retention. The enrollment of pediatric subjects in clinical trials has the additional complexity of family dynamics, legal status, and requirements of informed consent and assent. Medpace understands that successfully adhering to regulations and ethical standards in study conduct requires a comprehensive understanding of the unique concerns raised by ethics committees, investigators and parents.

Ethical Considerations

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. The enrollment of pediatric subjects in clinical trials has the additional complexity of family dynamics, legal status, and requirements of informed consent and assent. The vulnerability of children demands special expertise with regard to pediatric investigators and awareness of the intricate ethical issues associated with conducting these trials.
Regulatory expertise to navigate global challenges for study start-up

Globally, clinical research 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.

Pediatric study regulatory standards

- ICFs and Assents – different countries, different Ethics Committees (EC), different requirements
- ECs may want confirmation investigators are experienced with pediatrics
- Evaluation of burden to patients

Feasibility and site relationships to ensure enrollment goals are met

Subject enrollment and activity may occur at a slower rate across investigative sites. The Medpace team’s experience working with academic groups and pediatric research networks has been fundamental to success in past studies. Medpace has the necessary relationships to conduct feasibility studies and efficiently resource programs allowing for Sponsors to project milestones in these challenging studies.

Patient recruitment and retention to ensure milestones

Pediatric trials in rare diseases can have particular challenges in recruiting and retention. Specific recruitment challenges in trials involving children and adolescents are related to the unique emotional issues for children with disease, social/family dynamics, as well as practical issues such as scheduling, transportation, drug administration, storage, and compliance. Knowing that regulatory timelines at some academic institutions are often substantially longer than those that use centralized IRBs, the Medpace study team works to develop relationships with the sites as soon as we begin work.

A collaborative effort – Key opinion leaders paired with operational teams with experience in conducting innovative pediatric trials make Medpace the first choice in pediatric studies

Over the years, Medpace has amassed scientific, regulatory and operational experts with a passion for clinical development in rare diseases. Because rare disease goes hand in hand with pediatric studies, our depth of knowledge and experience in working with these special populations further enhances our ability to conduct projects in this area. Medpace brings a depth of experience and expertise that is widely applicable to a variety of clinical studies in rare disease.

These pediatricians have robust experience navigating pediatric studies covering metabolic, endocrinologic, cardiologic, infectious diseases and hematologic/oncologic indications. Medpace physician involvement plays a key in any study success, serving in both consultation regarding study design and protocol development with medical monitors, embedded for team education and support in each stage of a study. The medical supervision and decision making authority at the level of the CRO ensures subject safety while allowing a reduced level of Sponsor oversight, review, and training.
Physician Led: Present at all levels of study management

- Consultation on development program
- Protocol design
- Training and support of Medpace team
- Protocol interpretation
- Interpretation of SAEs, safety reporting, and narrative generation
- Study reports, regulatory submissions, and manuscripts

Hematology and Oncology

Cardiology

Pediatricians for physician led studies

Frank Smith, MD, FAAP, FACP

Pirouz Shamszad, MD, FAAP, FACC

Gretchen Williamson, MD, FAAP

Brian Murphy, MD, MPH

Endocrine

Infectious Disease
Brian Murphy, MD, MPH
Vice President, Medical Affairs, Infectious Diseases and Vaccines

Dr. Brian Murphy is board certified in infectious diseases, internal medicine, and pediatrics and has over twelve 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

Pirouz Shamszad, MD, FAAP, FACC
Medical Director, Cardiology

Dr. Pirouz Shamszad is board-certified in both cardiology and pediatrics and has extensive experience in the clinical development industry.

Experience Summary
- Significant clinical exposure in the natural history and management of critically ill patients hospitalized with various forms of congenital and acquired cardiac disease
- Previously: Attending Cardiologist in the Cardiac Intensive Care unit at Cincinnati Children’s Hospital
- Fellow of the American Academy of Pediatrics and American College of Cardiology
- Active member of the American Heart Association and Pediatric Cardiac Intensive Care Society

Education Summary
- Doctor of Medicine, George Washington University, School of Medicine
- Residency & Cardiology Fellowship, Baylor College of Medicine, Texas Heart Institute, & Texas Children's Hospital
- Advanced Fellowship, Cardiac Intensive Care Unit, University of Cincinnati – College of Medicine & Cincinnati Children's Hospital
Franklin O. Smith III, MD, FAAP, FACP  
Vice President, Medical Affairs, 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
- Over three decades of academic clinical practice in pediatric hematology/oncology and adult hematopoietic stem 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
- More than 170 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

Gretchen Williamson, MD, FAAP  
Senior Medical Director, Endocrine

Dr. Gretchen Williamson is a board-certified pediatrician that specializes in metabolic disorders.

Experience Summary
- Extensive medical monitor experience in diabetes (Type I and II), hyperlipidemia/dyslipidemia, and orphan indications
- Expertise in protocol development for clinical trials evaluating hypogonadism, pancreatic insufficiency, growth hormone deficiency, muscular dystrophy, rheumatoid arthritis, stem cell therapies

Education Summary
- Doctor of Medicine, Northeast Ohio Medical University
- Post-graduate training in pediatric endocrinology from Cincinnati Children’s Hospital.
- Residency in Pediatrics, Penn State University, Hershey Medical Center
Specific Indications covering Rare Disease and Pediatrics:

- Acromegaly
- Acute Otitis Media (AOM)
- Acute Bacterial Conjunctivitis
- TTR Amyloidosis
- Adrenocortical carcinoma (ACC)
- Community-Acquired Bacteria Pneumonia (HAP)
- Cushing’s Syndrome
- Cystic Fibrosis
- Duchenne’s Disease, Muscular Dystrophy
- Type II Diabetes
- Eosinophilic Esophagitis
- Familial Hypercholesterolemia
- Fibrodysplasia
- Gastroparesis
- Glomerulonephritis
- Growth Hormone Deficiency
- Hospital Acquired Pneumonia (HAP)
- Intra-abdominal Infections in Children
- Niemann Pick Type C disease
- Nonsense Mutation Dystrophinopathy (Duchenne’s)
- Neuroblastoma
- Ossificans progressive (FOP or Stoneman’s Disease)
- Renal vasculitis
- Respiratory Syncytial Virus (RSV) in lung transplant patients
- Severe Pertussis Syndrome (Whooping Cough)
- Uncomplicated Skin Structure Infections
- Ventilator-Associated Pneumonia (VAP)

About Medpace:

Medpace is a scientifically-driven, global full-service clinical research organization (CRO) providing Phase I-IV clinical development services for drug, biologic, and device programs. Medpace’s physician-led, high-science, and disciplined operating approach leverages regulatory and therapeutic expertise to accelerate the global development of safe and effective medical therapeutics across all major areas including oncology, cardiovascular, metabolic/diabetes, infectious disease, and neuroscience. Learn more at Medpace.com.