THE POWER OF X

MEDPACE

RARE DISEASE

Experts. Experience. Execution.

The Power of X in Rare Disease

Experts:

- Committed cross-functional team of Rare Disease experts comprised of doctors, project managers, regulatory consultants, and late phase specialists to orchestrate these complex studies
- Analytics driven patient recruiting teams using their extensive experience across a range of therapeutic areas to execute strategies designed specifically for your clinical trial needs

Experience:

- Conducted over 40 global trials
- Includes Phase I-IV
- Experience in conducting trials in over 15 different indications
- Our experts have years of experience working with rare diseases and orphan indications

Execution:

- Full-service approach to drug development - visit medpace.com for a list of comprehensive services
- Strong relationships with key industry investigative sites and KOLs

Challenges Conducting Rare Disease Studies

Conducting rare disease studies pose unique challenges in terms of study design, regulatory requirements, site identification, patient recruitment, operational logistics, and real world evidence data for post-marketing. Due to its complexity, these studies demand a CRO with experts, experience, and exceptional execution in rare disease and orphan indications.

Delivering a Full Service Solution

Rare Disease studies are complex by nature. Delivering excellence across all areas is second nature for Medpace. The Medpace study approach is to engage early, providing strategic thinking at the outset across study feasibility and start up, regulatory considerations, and site and patient recruitment. Medpace study teams are adept and trained to understand complex global studies having worked on difficult protocols with rare patient populations for many years.

Focused Rare Disease Teams Deliver Results

- Committed cross-functional team of Rare Disease experts comprised of doctors, project managers, and regulatory consultants to orchestrate these complex studies
- Board-certified Pediatricians with backgrounds treating pediatric patients with Rare Disease / Orphan Drug conditions for pediatric projects
- Global expertise in varied regulatory issues critical for Rare Disease projects
- ClinTrak™, Medpace proprietary Study Management system, providing decision support for all aspects of a project including managing Late Phase Patient registries

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Advanced Therapy Expertise

Advanced Therapy Medicinal Products (ATMPs) are rapidly demonstrating clinical effect in an increasing number of diseases and conditions that were previously out of reach of traditional drugs and biologics. Medpace offers sponsors comprehensive solutions to the unique and rapidly evolving clinical, operational and regulatory challenges of cellular, tissue and gene therapy medicines. Our work with innovators, regulators, key opinion leaders and investigators in this area gives us unique insight into the challenges and hurdles to overcome as we advance your product through the development process.

Regulatory Excellence

Orphan medicines are those intended to treat rare diseases and represent the focus of regulatory agencies in driving state of-the-art innovation in this area. Regulatory bodies define rare diseases as those that are life-threatening or chronically debilitating conditions. Medpace has developed experienced project teams for these complex studies. Integrated processes regarding site relationships with access to patient registries to drive patient enrollment, broad understanding of patient advocacy issues and organizations for support, and noted physicians and pediatricians are critical to a project's success.

A Different Take on Regulatory Submissions

The regulatory environment is complex. Many countries have few existing investigative sites for rare disease patients. Based on a majority of local EC/IRB sites and multiple review committees due to patient age or novel therapies, patients may request to access existing sites across borders. Medpace regulatory teams understand these regulatory complexities.

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Different Approaches for Specific Needs

Medpace employs a number of different approaches to study start-up, feasibility and patient recruitment in ultra rare diseases. Successful rare disease studies demand key differences as compared to typical trials with regard to investigative site identification, regulatory issues, and patient recruitment and retention. As a leader in conducting studies in ultra-rare disease, Medpace has developed best practices to deliver innovation at each step of process.

Targeted Site Selection for Rare Disease Patients

Rare Disease studies are very different from typical trials. Medpace Rare Disease teams use unique tools to conduct site selection, ensuring superior patient recruitment for these difficult to recruit studies.

Novel Patient Group Recruitment Approaches

Medpace works with Rare Disease advocacy groups, participating in advocacy events, regularly updating those communities and posting information about upcoming studies. Medpace sponsors pre-screening programs and registry partnerships. In addition, Medpace has developed a referral network of investigators who may refer patients from other institutions for Medpace studies.

Medpace's Patient Centered Approach

Rare Disease Patients face untold obstacles in their daily lives and treatments. Minimizing patient burden is a key success component in developing a committed group of patients for these programs. Some of the strategies for a patient focused approach include:

- Recommending a sequence of study procedures
- Tailoring assessments to minimize patient discomfort
- Strategically planning a positive patient experience and reduce patient and caretaker disruption
- Facilitating family travel across small and great distances

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Experience List

Medpace has conducted more than 40 global trials covering Phase I-V, involving 4,500 patients including these rare disease indications.

- Acromegaly
- Adrenocortical carcinoma (ACC)
- Cushing's Syndrome
- Cystic Fibrosis
- Duchenne Muscular Dystrophy (DMD)
- Eosinophilic Esophagitis
- Familial Hypercholesterolemia
- Fibrodysplasia
- Gastroparesis
- Glomerulonephritis
- Growth Hormone Deficiency
- Muscular Dystrophy
- Niemann Pick Type C disease
- Ossificans progressive (FOP or Stoneman's Disease)
- Renal vasculitis
- Respiratory Syncytial Virus (RSV) in lung transplant patients
- Tinnitus
- TTR Amyloidosis

Scientifically-Driven for Complex Projects

Led by noted experts across hematology, oncology, cardiovascular, metabolic, neuroscience and infectious disease. Many of our physicians are also board certified pediatricians.

Frank Smith
MD, FAAP, FACP

Terence Eagleton,
MB BS, (Hons)

Gretchen Williamson
MD, FAAP

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

Terence Eagleton, MB BS, (Hons)
Senior Medical Director
Dr. Terence Eagleton is an experienced physician with years of experience with rare disease, respiratory, and infectious diseases.

Experience Summary
- Extensive experience in rare and ultra-rare diseases
- Numerous manuscripts published in international peer-reviewed medical and scientific journals
- General surgical training (with a significant neurosurgical component) and experience with a wide variety of therapeutic areas and clinical phases

Education Summary
- Bachelor of Medicine and Bachelor of Surgery (Honors), University of London
- Bachelor of Science in Physiology (Honors), University of London

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

About Medpace

Medpace is a scientifically-driven, global, full-service clinical contract research organization (CRO) 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. Headquartered in Cincinnati, Ohio, Medpace employs approximately 2,500 people across 35 countries.