



METABOLIC AND ENDOCRINE RARE DISEASE

Experts. Experience. Execution.

Medpace Advantage - Metabolic and Endocrine Rare Disease

Rare Disease studies pose a different challenge in terms of project management, site selection, regulatory requirements, patient enrollment and retention. Rare disease – defined in the US as 1 in 200,000 patients - demand a CRO with innovative strategies to manage these programs. In particular, Rare Disease studies demand a CRO with expertise in enrolling rare disease patients and maximizing site selection to create realistic study feasibility.

Designing protocols and executing study design for scarce patient populations including pediatric patients who comprise 50% of these populations require medical doctors and pediatricians with specialized expertise. In addition, study design inclusive of a late phase component with access to Rare Disease patient registries is a critical tool.

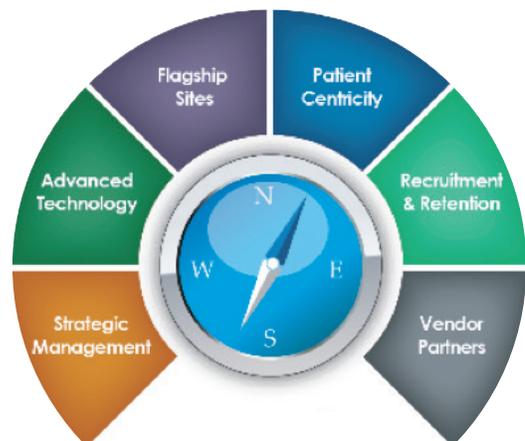
Innovative Approach for Rare Disease and Orphan Drug Projects

- Committed cross-functional team of Rare Disease experts comprised of doctors, project managers, regulatory consultants, and late phase specialists to orchestrate these complex studies
- Data Analytics: At the onset, Medpace Rare Disease teams are able to understand complex studies, and work with our existing and potential site partners to ensure study feasibility is thorough
- Study Start-up: Integrated teams to quickly engage with sites, to launch complex studies effectively and efficiently
- Site Relationships: Medpace utilizes existing flagship sites to accelerate study start up. Medpace has key site relationships and partners with advocacy groups to support enrollment needs
- Regulatory teams work hand in hand with study start with regard to study submissions, country guidance, and other considerations for critical regulatory issues in supporting our customers through the global orphan designation pathway

Solutions in Patient Recruiting- Navigate

Patient Recruitment enrollment is critical to your study success. Rare Disease studies depend upon expertise in understanding the challenges these patients face. Relationships with advocacy groups is a critical factor for success.

Medpace understands the challenge. The Navigate patient recruitment strategic solution is a multi-dimensional model enabling innovative, comprehensive, and customized recruitment and retention strategies that identify, recruit, and retain members of specific patient populations.



Rare Disease and Orphan Drug Experience

Medpace has conducted over 15 studies in Metabolic – Endocrine Rare Disease indications involving over 900 patients over 350 sites globally. Medpace has experience in the following indications: Acromegaly, Familial Chylomicronemia Syndrome, Lipodystrophy, Homozygous Familial Hypercholesterolemia, Nonsense Mutation Mucopolysaccharidosis, and Cushings Syndrome.

Medical Expertise in Pediatrics and Rare Disease:

Pediatrics

Gretchen Williamson, MD, FAAP

Medical Director

Dr. Williamson completed residency training at Penn State Hershey Medical Center and has post-graduate experience in pediatric endocrinology from Cincinnati Children's Hospital. She has clinical research experience in diabetes, hypercholesterolemia, hypertriglyceridemia, and multiple orphan indications (including familial chylomicronemia). She also has protocol development experience for numerous therapeutic areas, including rheumatoid arthritis, hypogonadism, pancreatic insufficiency, growth hormone deficiency, and stem cell therapies.

Endocrine and Metabolism

Phillippa Miranda, MD

Senior Medical Director

Dr. Miranda received her M.D. from Duke University School of Medicine and completed her residency in Internal Medicine and her fellowship in Endocrinology at Duke. She served on the faculty in the Division of Endocrinology at Duke University Health System for five years prior to transitioning to industry. Dr. Miranda is board certified in Endocrinology, Diabetes, and Metabolism. Her therapeutic expertise covers a wide range of metabolic indications with significant medical monitoring experience in the areas of type 2 diabetes, type 1 diabetes, and obesity. Dr. Miranda has over seven years of experience conducting clinical research studies with a large CRO and is well versed in the conduct of Phase 2 and 3 studies in endocrinology.

Douglas Lee MB BCh, MRCP, MBA

Senior Medical Director

Dr. Lee is an experienced drug developer with about 20 years of experience in both clinical medicine and drug development, with therapeutic expertise in Endocrinology and Metabolic Disease. Dr. Lee is currently a clinical reviewer for the journal Diabetes, Obesity and Metabolism. Prior to joining Medpace, Dr. Lee was the Global Senior Medical Director for a large pharmaceutical company where he designed, oversaw and executed global development plans involving early and late phase assets. In 2012 -13, he led a team that gained Marketing Authorization Approval (MAA) for 3 Endocrinology and Metabolic products, under the European Centralized Procedure. Dr. Lee received his Bachelor of Medicine, Bachelor of Surgery at the Queens University Medical School in the United Kingdom (UK) and achieved his Membership of the Royal College of Physicians in UK. He also earned his MBA from the Imperial College London in 2013.

Alicia Weeks, MD*Medical Director*

Alicia Weeks is an experienced Clinical Endocrinologist with a former academic clinical practice and translational research experience in Type 2 Diabetes. As a Medical Director at Medpace, she provides medical management and expertise to all phases of studies and clinical trials, as well as assists with new business development through participation in proposal and Sponsor meetings. Dr. Weeks gained experience from a dual appointment as a clinical instructor at the University of Wisconsin Hospital & Clinics and as a Research Fellow at the William S. Middleton Memorial Veterans Hospital after earning her medical degree from the Wright State University Boonshoft School of Medicine. Dr. Weeks completed her residency at the Kettering Medical Center and an endocrinology fellowship at the University of Wisconsin. There she was awarded the Dickie Research Award for her significant research contribution toward advancing the field of medicine.

Rare Disease**Terence Eagleton, BSc., MD.***Senior Medical Director, Medical Affairs*

Dr. Eagleton is a UK physician who trained as a general surgeon, with a particular interest in trauma management and critical patient care. Dr. Eagleton has subsequently worked as a senior pharmaceutical physician in the biopharma industry for over 17 years in global clinical research, and medical affairs in a wide variety of therapeutic areas. He has extensive experience and achievement in international drug development across Phase I to IV, with a specific focus and expertise in the clinical development of therapies to treat rare and ultra-rare metabolic and endocrine disorders.

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.