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**

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's patient recruitment platform - IntelliPACE™ - provides a strategic blueprint for optimal patient recruitment. Medpace’s data-driven, experienced refined approach aligns the target population with motivated and experienced Investigators to create a precise and effective recruitment strategy.

**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.
**Terrence Eagleton, BSc., MD**  
**Senior Medical Director, Medical Department**

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.

**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 of 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.

**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.

**Medpace has experience in the following indications:** Acromegaly, Familial Chylomicronemia Syndrome, Lipodystrophy, Homozygous Familial Hypercholesterolemia, Nonsense Mutation Mucopolysaccharidosis, and Cushings Syndrome.
EXECUTION

DRIVING EFFICIENT AND CONSISTENT DATA FOR GLOBAL STUDIES

Achieve quality results, meet deadlines, and maximize efficiencies by partnering with a CRO that excels in execution. Medpace demonstrates a commitment to quality and a dedication to conducting full-service studies in an exacting manner to produce the highest quality results.

KEYS TO SUCCESSFUL EXECUTION

- **Committed Teams:** Our team is with you from project initiation to completion. As a result, we typically develop better team dynamics based on trust and respect
- **Resourcefulness:** Medpace promotes a culture of problem-solving. Our global operational staff proactively identify potential issues and communicate solutions, ensuring your sites and studies are managed effectively and efficiently
- **Regulatory Support:** Medpace offers a dedicated global regulatory submissions team as well as global regulatory affairs for comprehensive support

THE MEDPACE MODEL

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.

FULL-SERVICE CLINICAL DEVELOPMENT

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.