MEDPACE

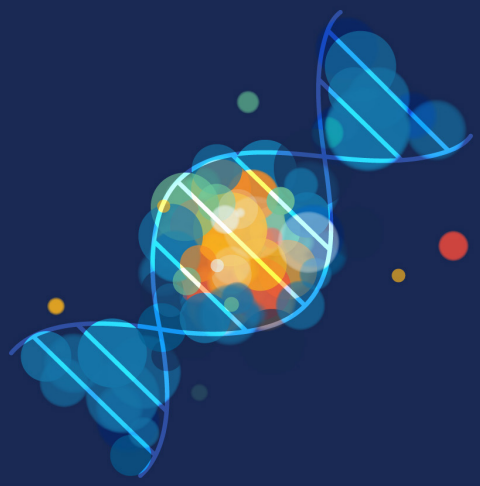
RARE DISEASE & ORPHAN INDICATIONS CLINICAL DEVELOPMENT

Rare Disease clinical trials present a unique set of challenges in terms of clinical trial design, regulatory strategy, site selection, patient enrollment, and project management. Rare disease studies require a CRO experienced in scarce patient populations and innovative strategies to manage these programs.

Medpace has experience in the rare disease and orphan drug space having conducted more than 250 trials involving 23,000 patients at 7,350 sites in 67 countries across indications, spanning all ages, and in a spectrum of therapeutic areas, including, but not limited, to metabolism, neuroscience, hematology/oncology, cardiology, nephrology, immunology, and ophthalmology.

EXAMPLE INDICATIONS

- Acromegaly
- Adrenocortical carcinoma
- Cushing's Syndrome
- Cystic Fibrosis
- Duchenne Muscular Dystrophy
- Eosinophilic Esophagitis
- Familial Hyper-cholesterolemia
- Fibrodysplasia
- Gastroparesis
- Glomerulonephritis
- Growth Hormone Deficiency
- Muscular Dystrophy
- Niemann Pick Type C disease
- Ossificans progressive
- Renal vasculitis
- Respiratory Syncytial Virus in lung transplant patients
- Tinnitus
- TTR Amyloidosis



EXPERTS

- Cross-functional rare disease team of experts comprised of doctors, advanced clinical practitioners, operations, regulatory, laboratory, and imaging
- In-house pediatricians with backgrounds treating pediatric patients with rare disease/orphan drug conditions
- Regulatory footprint to guide and manage studies at local and global levels
- Analytics-driven patient recruiting teams specialized in scarce patient populations

EXPERIENCE

- Deep understanding of patient advocacy issues and organizations for added support
- Adept in conducting global complex studies with multifaceted requirements for every stage of clinical development
- Recognized leader in conducting trials using gene and cell therapies (Advanced Therapy Medicinal Products - ATMPs)

EXECUTION

- Well-established and strong relationships with relevant KOLs
- Proactive patient recruitment and retention planning tailored to global rare disease studies
- Site relationships with access to patient registries to drive patient enrollment
- Study management technology to support all aspects of a trial
- Thorough feasibility processes assess interest in trials and volume of specific populations
- Wholly-owned central and bioanalytical labs, imaging and ECG core labs deliver integrated efficiency

TARGETED SITE SELECTION

Rare disease studies are very different from typical trials. Medpace rare disease teams use innovative data-driven tools to plan and conduct site selection, ensuring superior patient recruitment for these difficult to recruit studies.

PROACTIVE PATIENT RECRUITMENT

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.

PATIENT-CENTERED APPROACH

Rare disease patients face untold obstacles in their daily lives and treatments. Minimizing patient burden is a key component in recruiting 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 to reduce patient and caretaker disruption
- Facilitating family travel across small and great distances

REGULATORY EXCELLENCE

Medpace works collaboratively with Sponsors and regulatory bodies to identify and navigate the best pathways for approval in key regions including the US, Europe, and Asia-Pac. Understanding the regulatory nuances and guidances for each region is critical to developing sound strategies. Medpace's Regulatory Affairs has extensive experience and success in this area and can help Sponsors with early and on-going support.

GLOBAL CENTRAL LABS

Our wholly-owned central laboratory, is fully-integrated with our CRO services. Medpace combines our in-house trial management expertise with laboratory services in a well-coordinated and streamlined process. Medpace has extensive experience in rare disease trials and can provide global central laboratory analysis of safety labs and manage sample collection, processing and distribution of biopsy samples, as well as sample storage for future analyses.

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.

WE CAN'T SIMPLIFY CLINICAL DEVELOPMENT – BUT WE CAN EXECUTE IT SEAMLESSLY.

MAKING THE COMPLEX SEAMLESS®