MEDPACE

RARE DISEASE

Experts. Experience. Execution.

Discover the POWER OF X

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

Rare Disease and Orphan Drug research is challenging.

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

Medpace offers an innovative full service approach for these unique 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 work quickly to engage with sites to launch complex studies effectively and efficiently

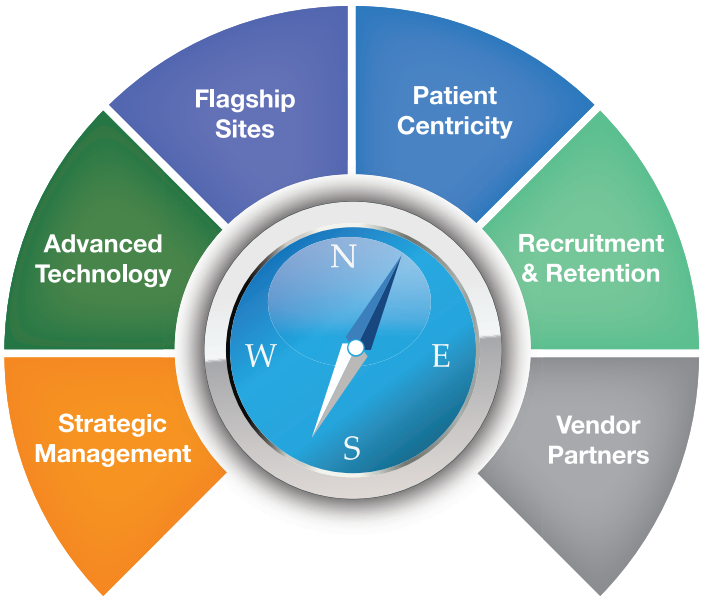
Site Relationships: Medpace utilizes existing flagship sites to accelerate study start up. Medpace has key site relationships and partners with advocacy groups to support enrollment needs.

Regulatory Expertise for Advanced Therapies: With regard to study submissions, country guidance, and other considerations for critical regulatory issues, regulatory teams work hand in hand with study start up. We support our customers through the global orphan designation pathway.

Medpace 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 deep experience in the rare disease and orphan drug space having conducted more than 40 global trials covering Phase I-IV, involving 4,500 patients. These studies include more than 15 indications of rare diseases.

Rare Disease Implication Sample Experience

- Acromegaly
- Adrenocortical carcinoma (ACC)
- Cushing's Syndrome
- Cystic Fibrosis
- Duchenne Muscular Dystrophy (DMD)
- Eosinophilic Esophagitis
- Familial Hyper-cholesterolemia
- 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

Who We Are

Medpace is a global full-service clinical research organization providing Phase I-IV core development services for drug, biologic, and device programs. With medical and regulatory expertise in multiple therapeutic specialties, Medpace has assembled therapeutically focused teams to execute at every level of the company's operations, providing complete and seamless drug and device development services.

MEDPACE

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