



Orphan Reach™ - Emmes' Rare Disease Center

A dedicated division for the advancement of rare disease research and specialized clinical trial design

Emmes is known for excellence in clinical trial support, and that stems from our roots in statistical science and unwavering dedication to delivering superior research support as a CRO since 1977. Emmes has a long history of supporting rare diseases across a wide array of therapeutic areas in both adult and pediatric populations.

With approximately 7,000 rare diseases affecting over 300 million people worldwide, clinical trial research in rare diseases is a top priority. Currently only 5% of rare diseases have an approved treatment even though the number of FDA orphan drug and biologic approvals has grown exponentially since passage of the Orphan Drug Act. We strive to meet this recognizable need and are at the forefront of groundbreaking research for these small, yet collectively large populations.

Emmes' commitment to rare disease research is shown by Orphan Reach - its dedicated Rare Disease Center. Our goal is to accelerate the clinical development of urgently needed new treatments for rare diseases. Our mission is to provide a tailored approach to designing and conducting rare disease studies in order to expedite the clinical development of orphan drugs and ultimately improve the quality of life of patients and their families.



Fully dedicated
rare disease
expert team

More than 60
rare disease trials
conducted

Operating in more
than 70 countries

Highlights Include:

Rare disease studies are challenged by small sample sizes, sparse and geographically remote patient populations, difficulty in diagnosis, and preponderance of diseases with a genetic etiology. We offer a lengthy history supporting global research in cell and gene therapies as well as rare diseases across a wide array of therapeutic areas. Expertise in bioinformatics and adaptive/alternative study designs has enabled our clients to conduct even the most challenging rare disease studies. Our experience in novel clinical trial designs and approaches for rare diseases include:

- Single-arm trials with internal or external controls
- Adaptive study designs
- Umbrella/Basket/Platform trial designs
- Many-to-one matching designs
- Randomization enrichment approaches
- Simulation and modeling approaches
- Patient-centric approaches for outcomes and statistical analyses

Patient recruitment and retention are the cornerstones of a successful clinical trial in rare diseases. Orphan Reach uses a dynamic infrastructure to allow access to patients with rare

diseases in more than 70 countries. Our global teams coordinate efforts in each of their local territories and contribute their rare disease expertise to accelerate access to new treatments in a wide range of orphan therapeutic areas.

While providing full-service CRO support for rare disease studies we offer global clinical development services that allow clients to take their precious IMP from early trials in patients to Marketing Authorization. Our clients appreciate our refined process in dealing with unique medical, scientific, clinical and commercial challenges.



Contact us at info@emmes.com to learn more about our experience, or visit www.orphan-reach.com