Emmes

Rare Disease

Collaborate with our dedicated team of experts, offering strategic leadership and tailored support for your rare disease clinical trials.

Emmes' Rare Disease team harness more than 30 years of orphan drug development expertise, spanning work in both public and private sectors. We offer a patient focused approach, with an emphasis on efficient, cost-effective trial designs that turn patient voices into better data.

Emmes provides full-service CRO support for rare disease clinical trials as well as offering global clinical development services.

Services Offerings

- Clinical Monitoring
- Project Management
- Quality Assurance
- Pharmacovigilance
- Biostatistics & Bioinformatics
- Strategic regulatory expertise
- Patient tailored study design
- Novel Outcome Development

Modernizing clinical research with tech & AI

At Emmes we are modernizing and automating clinical research across the full spectrum of clinical trial activities to operate faster, more efficiently, and with higher quality. We are the industry's first native digital and AI specialty CRO built on a proprietary technology and AI platform.

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What sets us apart?

Benefits of our services

Engaging the rare disease patient community – Emmes' strategic patient advocacy team has experience supporting patientrecruitment for rare disease trials through community outreach, for example having Subject Matter Experts bringing their lived experiences to rare disease trials.

Innovative endpoint design and biostatistics- Emmes Endpoint Solutions enables innovative endpoints and validated qualitative research protocols, for example concept elicitation approaches applicable across different rare disease indications and the first Duchenne Muscular Dystrophy endpoint accepted into FDA's COA Qualification Program.

Established relationships with Sites, Investigators and Patient Advocacy Groups – adds efficiencies to study start up and increases likelihood of accelerating timelines for recruitment goals.

Unique approach to trial operations – utilizing our flat structure to provide flexibility and a clear escalation pathway for clients creates a true collaborativeapproach to trial execution and problem solving.

Highlights Include

Developing the first Duchenne Muscular Dystrophy endpoint to be acceptedinto FDA's COA Qualification Program – the Duchenne Video Assessment (DVA) tool- leveraging strategic partnerships in the Duchenne community to successfully complete patient recruitment in a decentralized trial setting.

Incorporating bespoke strategies to support client enrollment efforts in a limited patient population – resulting in exceeding enrollment expectations through activation of sites in two backup countries, contributing 15% of the overall recruitment numbers in a Primary Mitochondrial Myopathy study.

Leveraging innovative trial design- managing two first-in-human gene transfer studies since 2014 in Stargardt's Disease and Usher's Syndrome.

Enhancing feasibility and trial success- managing trials with a patient focused pathway incorporating patient/family preferences, burden of participation and real world impact to enhance the likelihood of trial success.

Key Statistics

100+

Global access to local resources in excess of 100 countries

290+

More than 290 projects in the rare disease space

55+

Supported over 55 Biopharma Clients

9

Completed projects across nine rare disease areas

For additional information on our Rare Disease services, please visit:
www.emmes.com

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