WORLDWIDE
CLINICAL TRIALS

IS YOUR CRO THE RIGHT FIT FOR YOUR RARE OR ORPHAN DISEASE TRIAL?

WORLDWIDE CLINICAL TRIALS

RARE DISEASE PORTFOLIO

- Alzheimer's Disease (Early Onset)
- Amyotrophic Lateral Sclerosis (Lou Gehrig's)
- Angelman Syndrome
- Bullous Pemphigoid
- Congenital Adrenal Hyperplasia
- Cutaneous T Cell Lymphoma
- Cystic Fibrosis
- Familial Mediterranean Fever (FMF)
- Gaucher's Disease
- Glioblastoma (GBM)
- Growth Hormone Deficiency
- Hepatocellular Carcinoma
- Hormone Replacement
- Hypercholesterolemia
- Hypertension, Pulmonary
- Idiopathic Pulmonary Fibrosis
- Idiopathic Thrombocytopenia Purpura
- Infantile Spasms
- Juvenile Rheumatoid Arthritis
- Lambert-Eaton Myasthenic Syndrome (LEMS)
- Leukemia, Acute Myeloid
- Lower Limb Spasticity
- Metabolic Conditions
- Mucopolysaccharidoses
- Multiple Sclerosis
- Multiple System Atrophy
- Muscular Dystrophy
- Myasthenia Gravis
- Myelodysplastic Syndrome
- Pain (Neuropathic)
- Pemphigus Vulgaris
- Restless Legs Syndrome (RLS)
- Sickle Cell Disease
- Stomach Cancer
- Ulcerative Colitis

CLINICAL ASSESSMENT TECHNOLOGIES

Accurately predicting site performance and GCP compliance is especially difficult in rare disease indications. Clinical assessments technologies oversee the integrity and clinical meaningfulness of your program's assessments. Features include:

- Corroboration of patient eligibility
- Custom-designed CAT plan to ensure consistent calibration of assessments
- Practical standards for rater credentials and certification
- Rigorous rater training
- Meticulous in-study data surveillance
- Database build monitoring
- Scale and translation management

FINDING RARE DISEASE PATIENTS IS DIFFICULT

The rare disease clinical research space is, by definition, fraught with unique challenges. Although the rate of advancement in the field is increasing, recent assessments indicate that more than 90% of rare diseases still have no approved treatments.[1] The low patient populations inherent with rare diseases necessitate enhanced recruitment strategies and specialized considerations related to geographic location and investigative site feasibility. Comorbid conditions add to the complexity of diagnosis and assessment, often requiring coordination among multiple specialties.

Adding to these challenges is the reality that 50% of rare diseases affect pediatric patients, and many are fatal in infancy and childhood. In addition to the special needs of pediatric patients and their caregivers, researchers must take into account the physical and emotional development of the pediatric patient over the course of study, as well as any potential changes in patient attitudes about their role as study subject.

A HOST OF OPPORTUNITIES

Opportunities for researchers have never been more promising, especially considering the growing motivation among policymakers, regulators, and investors to increase advancements in the diagnosis and treatment of rare diseases. Organizations that can navigate the complex rare disease space have the potential to make groundbreaking progress.

Offices in Emerging Markets for Access to Hard-to-find Patients Across 60+ Countries

[1]Source: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5759721/

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CONNECTIONS FOR EASIER RARE DISEASE RESEARCH

Our experience working with research networks, pediatric hospitals, specialized treatment centers, and academic institutions benefits your rare disease program. We connect with patient organizations and enlist key opinion leaders around the world to assist in the vetting of protocol designs, site identification, and patient recruitment.

For example, patient advocacy groups can support the recruitment of patients, yet they also can play an important advisory role during protocol drafting, specifically regarding feasibility of requested procedures in relation to possible patient and caregiver challenges. Additionally, disease-specific registries are particularly invaluable given the extraordinary insights available regarding patient pathway to diagnosis, patient management challenges encountered by families and physicians, and insights regarding clinically important changes that might be assessed during development.

Worldwide's commitment to "participatory" research, which proactively engages and connects all stakeholders – including patients, caregivers, scientists, clinicians, and communities – fosters a collaborative approach that supports the successful execution of your clinical research program.

WHAT CAN WORLDWIDE DO?

Biotech and pharmaceutical companies need a CRO with exceptional clinical trial expertise in the rare disease space, with the agility and flexibility needed to ensure patients' needs come first. Rare disease research calls for a nuanced balance of global reach, providing access to suitable patient populations and qualified investigative sites with a flexible but highly focused approach to patient recruitment, trial design, and project execution.

Here's how Worldwide can help:

Broad experience in rare diseases

Worldwide's professionals have been involved in many studies of rare or orphan diseases, providing design, execution and/or analysis services, including strategies that account for diversity in disease manifestation and progression.

Global infrastructure

Worldwide has a presence in more than 60 countries around the world, giving us access to remote patient populations. By maintaining close ties with our investigator sites, we have a boots-on-the-ground understanding of regional variations in regulatory requirements and standards of healthcare.

Strong relationships with regulators

The success of a clinical trial hangs on regulatory compliance. The success of a CRO relies on a consistent history of compliance combined with cooperative relationships with regulators. Worldwide's reputation for compliance and continued engagement with authorities gives us the ability to contribute regulatory knowledge in the rare disease space.

Patient-centric approach

Worldwide Clinical Trials recognizes the value of the patient's experience. Our experts can develop recruitment and retention strategies to promote the patient's willingness and ability to comply with protocol and even empower the patient to contribute to our knowledge base of their particular disease.

Industry and academic cooperation

In addition to our reputation among satisfied sponsor partners, Worldwide's history of collaboration with regulatory bodies, academic research organizations, and key investigators means we are known within the industry as a reliable research partner.

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THERE'S NO SUBSTITUTE FOR UNCOMMON EXPERTISE. MEET YOUR PARTNERS.

Ian Braithwaite, Ph.D.

Senior Vice President, Global Project Management

Ian Braithwaite brings deep industry and therapeutic experience to his role overseeing project management and operational delivery for Worldwide Clinical Trial's General Medicine, Rare diseases and Oncology business unit. He has full accountability for strategic development and execution of all projects conducted by these therapeutic areas, ranging from immunology/inflammatory diseases (rheumatoid arthritis, lupus, psoriasis, IBD, asthma, COPD, etc.) and rare diseases (for e.g., sickle cell disease, IPF, bullous pemphigoid, cutaneous T-cell lymphoma).

Dr. Braithwaite worked in clinical research for more than 26 years before joining Worldwide in 2015, including serving as Executive Vice President & GM at INC Research, and Global Therapeutic Area Head – General Medicine & Executive Director and Therapeutic Group Head Hematology/Oncology at PPD. Prior to joining the CRO industry, Ian was Director, Clinical Development at AstraZeneca Pharmaceuticals working primarily on Oncology and Immunology/Inflammatory Diseases. He began his career as a clinical research scientist at SmithKline & French after completing his academic studies.

Tricia McCain

Senior Director, Global Project Management

Tricia McCain brings multiple perspectives to the Rare Disease team, having been a Child Life specialist caring for the patients, a study coordinator at a children's hospital, monitor and project management of global, multi-service teams in rare disease and pediatric indications. She currently serves as Senior Director, Rare Diseases at Worldwide, providing support to clients and teams during all phases from development planning to proof in concept, including patient advocacy liaison. Her passion for helping others shines through her enthusiasm and willingness to not leave any stone unturned.

Michael Murphy, M.D., Ph.D.

Chief Medical and Scientific Officer

Dr. Murphy's professional career has spanned 25 years and his positions within the pharmaceutical industry emphasize the integration of medical and scientific acumen with operational excellence. He is board-certified in psychiatry and has a doctorate in pharmacology, with training at Tulane University, Stanford University and the Mt. Sinai School of Medicine.

Dr. Murphy worked with Dr. Cutler in articulating this vision for Worldwide when the company was established as a global CRO in 1995 and was responsible for consulting services for protocol and program design, and executive oversight for the execution, analysis and interpretation of clinical trials across multiple therapeutic areas.

His supervisory responsibilities as Chief Medical & Scientific Officer at Worldwide are international in scope and include the design and implementation of protocol feasibility assessments, protocol development for phases I–IV including non-interventional research, the provision of medical monitoring and drug safety services, medical writing, and coordination of rater certification and surveillance activities for clinical trial assessments.

Tatjana Odrljin M.D., Ph.D.

Executive Director, Medical Affairs

Dr. Odrljin oversees the Worldwide IMID/rare disorders medical affairs group and advises and supports all functional lines. She also provides medical support and management for clinical research studies as medical monitor (MM). Dr. Odrljin is the medical lead and manager for the Worldwide Clinical Trials medical affairs IMID/rare disorders team. She has worked extensively with key opinion leaders, in the fields of immunology, metabolic bone diseases, rare diseases (metabolic bone and renal) infectious diseases and patient advocacy groups.