



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



**WORLDWIDE  
CLINICAL TRIALS**



## RARE DISEASE PORTFOLIO

Acromegaly	purpura
Amyotrophic lateral sclerosis	Infantile Spasms
Cystic fibrosis	Juvenile idiopathic arthritis
Congenital Adrenal Hyperplasia	Lambert-Eaton myasthenic syndrome
Duchenne muscular dystrophy	Lupus Nephritis
Gastrointestinal stromal tumors (GISTs)	Multiple System Atrophy
Fabry disease	Mucopolysaccharidosis I, II, III, VI
Frontotemporal dementia	Mastocytosis
Gaucher disease	Myelodysplastic Syndrome
Growth hormone insensitivity syndrome	Pemphigus Vulgaris
Hemophagocytic lymphohistiocytosis	Phenylketonuria
Hemophilia A and B	Proteus/Pros Syndrome
IGF Deficiency	Sickle cell
Idiopathic pulmonary fibrosis	Thrombotic microangiopathy
Idiopathic thrombocytopenic	Von Willebrand disease
	Velocardiofacial syndrome, psychosis

## 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.<sup>1</sup> 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.



<sup>1</sup>Source: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5759721/>



## WORLDWIDE CLINICAL TRIALS

### 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.

### 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.

# WORLDWIDE RARE DISEASE EXPERIENCE

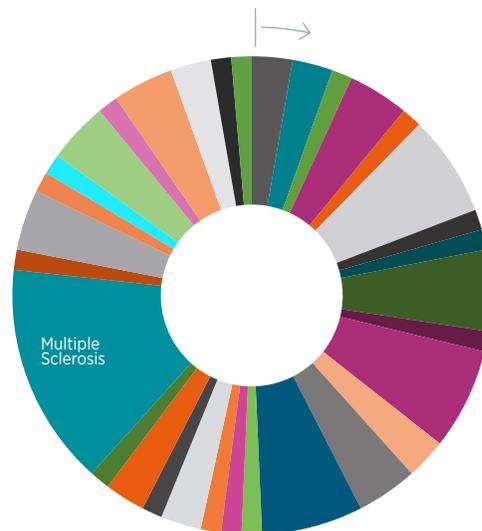


## Studies by Therapeutic Area

Therapeutic Area	Studies	Sites	Patients
Alzheimer's Disease	2	247	1,198
Amyotrophic Lateral Sclerosis (Lou Gehrig's)	2	28	450
Angelman Syndrome	1	2	70
Congenital Adrenal Hyperplasia	3	33	382
Cutaneous T-Cell Lymphoma	1	40	164
Cystic Fibrosis	5	214	609
Depression (Other)	1	13	108
Familial Mediterranean Fever (FMF)	1	3	50
Gaucher Disease	4	11	64
Glioblastoma (GBM)	1	7	45
Hepatocellular Carcinoma	5	64	212
Hypercholesterolemia	2	61	462
Hypertension, Pulmonary	3	185	257
Idiopathic Pulmonary Fibrosis	5	30	70
Idiopathic Thrombocytopenic Purpura	1	1	15
Infantile Spasms	1	44	190
Juvenile Rheumatoid Arthritis	1	16	30
Lambert-Eaton Syndrome	2	33	69
Leukemia, Acute Myeloid	1	10	6
Metabolic Conditions	2	45	97
Mucopolysaccharidoses	1	2	8
Multiple Sclerosis	11	303	1,894
Multiple System Atrophy	1	17	59
Muscular Dystrophy	3	40	114
Myelodysplastic Syndrome	1	2	15
Pain (Neuropathic)	1	2	32
Pemphigoid	3	22	31
Polymyalgia Rheumatica	1	3	50
Restless Legs Syndrome (RLS)	3	18	288
Sickle Cell Disease	2	28	73
Stomach Cancer	1	37	120
Ulcerative Colitis	1	36	490
<b>Total</b>	<b>73</b>	<b>1,597</b>	<b>7,222</b>

## Studies by Phase

Study Phase	Studies	Sites	Patients
Phase I	9	79	335
Phase II	26	381	1,897
Phase III	36	1,130	5,405
Phase IV-Interventional	2	7	85
<b>Total</b>	<b>73</b>	<b>1,597</b>	<b>7,222</b>



**73**

**Full-service\* rare disease trials conducted**

Worldwide has conducted 73 full-service\* rare disease trials globally, including sites in North and South America, Western Europe, Central and Eastern Europe, the Commonwealth of Independent States, and the Middle East and Northern African and Asia Pacific regions.

Q4 2018  
DATA VALID THROUGH 31-JANUARY-2019

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



Worldwide is committed to delivering uncommon value. In fact, we go above and beyond to deliver solutions and services that exceed your expectations. Our collaborative, customized project teams, with phase- and indication-specific expertise, develop successful strategies for even the most novel therapies.



**Leslie Wetherell**  
Executive Director, Project Management,  
Rare Disease/Oncology

Leslie Wetherell has over 20 years in the pharmaceutical industry, working in biotech, pharmaceutical, and the CRO industry. She has been with Worldwide Clinical Trials since June 2016. In her role, she is responsible for the portfolio of rare disease studies within the immune mediated inflammatory disorder therapeutic area.

Ms. Wetherell has extensive experience in hematology/oncology and rare disease in the following indications: breast cancer, NSCLC, prostate cancer, glioblastoma, solid tumor, colorectal cancer, pancreatic cancer, hepatocellular carcinoma, advanced cancer with bone metastases, GIST, supportive care (cdiff), cachexia, lymphomatous meningitis and late-stage lymphoma, CLL, SLL, multiple myeloma, Waldenström's macroglobulinemia, paroxysmal nocturnal hemoglobinuria, hemophilia A and B, von Willebrand disease.

She also has experience in the following indications: cardiovascular devices, dermal fillers, obesity, post-surgical pain, HIV, psoriasis, idiopathic pulmonary fibrosis, lupus nephritis.



**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 Odrliin, M.D., Ph.D.**  
Executive Medical Director, Immune Mediated Inflammatory Diseases,  
Rare Disorders Medical Lead, Medical Affairs

Dr. Odrliin 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. Odrliin 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.



**Tricia McCain**  
Senior Vice President, Global Project Management,  
Immune-Mediated Inflammatory Disorders (IMID), Rare Diseases & Oncology

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.