Make Your Move into Rare Disease Research Confidently

A Partnership Built for Your Success

Rare is Extraordinary

Rare disease research demands a unique approach — uncommon diseases require bespoke solutions. Ultimately, your success relies upon accessing specialized, proven rare disease research expertise that complements your team and delivers on your program objectives.

At Worldwide, we have assembled a skilled team and built an infrastructure to provide you with the personalized, patient-centered solutions that rare disease programs require.

In rare disease studies:

Every patient and data point collected is precious. In rare indications, the population is small, requiring extended care in the collection and preservation of every patient and data point.

Patients expect to be heard. We routinely partner with rare disease patient communities to make sure their perspectives are incorporated into the strategy and early stages of study design, driving successful recruitment and retention.

Logistics are complex. By nature of being rare, patients are geographically dispersed, requiring complex logistical solutions to support screening, enrollment, and retention.

Rare disease research is nuanced, but success is achievable alongside a team with the right blend of expertise, proven solutions, and lessons learned to inform the unique needs of your study.

Global Rare Disease Experience

210+ Projects

100+ Indications

6,100+ Site Activations

29,500+ Patients Enrolled

60+ Countries

Unmatched Methodology

Find out more about the distinct rare disease methodology we use to drive success for our sponsors →

Key Services for Your Program's Success

We look at a program holistically from each stakeholder's perspective to make sure your trial accomplishes what it sets out to do and meets the expectations of your patient population.

Service	Considerations	What does this entail?
Advocacy Engagement	What are the day-to-day challenges for your patients? What benefits do they need to consider enrolling?	• Focus groups • Surveys • Patient and caregiver interviews
Clinical Development Plan	What pre-clinical data are required? What endpoints have been/should be used to seek approval?	Our Clinical Methodology fellows, led by our founder, Dr. Michael Murphy, offer decades of experience in engaging with different divisions to support study planning and design.

Key Services for Your Program's Success (Continued)

Service	Considerations	What does this entail?
Regulatory Guidance & Planning	When/how to apply for orphan status?	We provide experts in FDA, EMA, and PMDA regulations.
Clinical Operations & Logistics	What is the most efficient operational strategy?	• Phase 1 through Phase 4 global operational support • Preferred vendor partnerships to support travel concierge services, family reimbursement, and hybrid decentralized study designs
Compassionate Use/Expanded Access Programs	What is the optimal approach for each development program? What is an appropriate approach to supporting patients after the trial ends?	• Global open-label extensions • Long-term follow-up studies • Expanded Access/Compassionate use programs ensure a satisfying experience for patients
Real-World Evidence	What will/can be used as a control data set?	• Operational expertise in registries and natural history studies • Access to medical claims data through our sister company, Trinity Analytics • Global operational strategy for access to historical medical records
Clinical Assessments Technology	How can we ensure all data collected are usable for analysis?	• We offer expertise in endpoint adjudication, mitigating placebo effect, and supporting quality COA/eCOA data capture.

Meet Our Rare Disease Strategy Leads

With a combined experience of more than 30 years, our rare disease team approaches every rare disease clinical trial with personalization and flexibility in mind.

Derek Ansel, MS, LCGC
Executive Director, Therapeutic Strategy Lead, Rare Disease

Meet Derek

Amy Raymond, PhD, PMP
Executive Director, Therapeutic Strategy Lead, Rare Disease

Meet Amy

Juliane K. Mills, MS, MPH
Senior Director, Therapeutic Strategy Lead, Rare Disease

Meet Juliane

Nathan Chadwick
Senior Director, Therapeutic Strategy Lead, Rare Disease

Meet Nathan

Worldwide Clinical Trials

Move Forward Confidently with Worldwide Clinical Trials

Your program's success relies on a customized approach from an experienced team. At Worldwide, our rare disease team has supported more than 210 projects around the globe, and we leverage these lessons learned in every study we support. From our deep understanding of the nuances of rare disease trials to our specialized rare disease methodology, you can be confident we're the right partner to help you make your move into the rare disease drug development landscape.

To learn more about our extensive experience in rare disease research and how we will partner with you, check out our **fact sheet** or **contact us today**.

Last Update: 05232024