

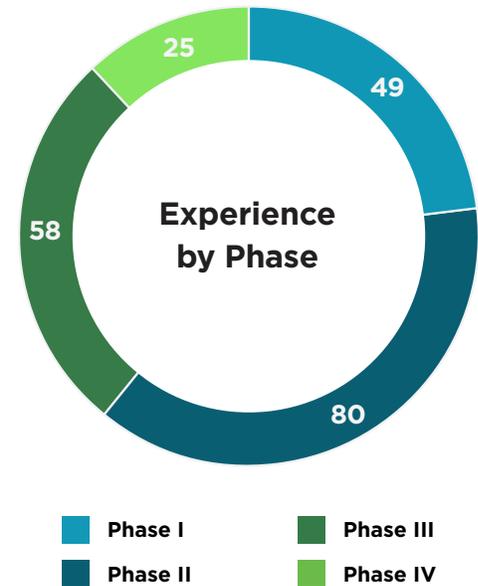
# Rare Disease Clinical Development at Worldwide Clinical Trials

## Rare disease clinical development is personal. For you, for us, for patients.

As a global CRO, Worldwide Clinical Trials is the right partner to help you overcome the complexities of rare disease development. We provide scientific expertise, therapeutically-aligned and agile operational teams, and a shared passion for bringing your new therapy to patients and their families. Everyone from the strategists you'll work with to the individual CRAs on your study has chosen to focus their professional expertise on rare disease communities within specific therapeutic areas.

## Specializing in Rare Disease Research Across a Range of Therapeutic Areas and Treatment Modalities

- Oncology
- Neuroscience
- Metabolic
- Respiratory
- Hematology
- Nephrology
- Gene Therapy
- Cell Therapy
- RNA Medicines



## 5 Year Full-Service Global Rare Disease Experience



**190**  
Trials



**95+**  
Indications



**6,000+**  
Site Activations



**25,000+**  
Patients



**60+**  
Countries

## Accessible Rare Disease Knowledge

Our dedicated team is well-versed in the nuances of rare indications studies, patients, and communities, taking a rare disease-specific strategic approach to your program. We also have rare disease regulatory expertise to support program advancements and help you navigate engagement with medicine agencies.

### Our Team Is Skilled in Supporting

- Endpoint selection and development to ensure data quality
- Rater training programs to reduce screen fails and improve data quality
- Risk reduction through strategic operational considerations, including country, site selection, and enrollment mitigations

### Corporate & Team Study Type Experience

- First-in-human (healthy volunteer & patient)
- Phase II-III studies
- Open-label extension studies
- Long-term safety follow-up studies
- Natural history studies & registries (disease & product)

## A Partnership for Your Success

We approach every rare disease study with [personalization and flexibility](#) in mind. From your company and study's unique needs to the individual needs of the patients, we take our time-tested knowledge of rare disease development and adjust it for each study, establishing an infrastructure capable of pivoting as needed. You'll also have direct access to our senior scientific and operational experts to help inform key decisions along the way.

*Rare Matters Worldwide* is our corporate commitment to partner with like-minded non-profit organizations in their pursuit of new cures and innovations for rare diseases.

Earlier this year, we announced our formal partnership with Every Cure to expedite the discovery of treatments for patients with rare diseases. Worldwide serves as a drug development and clinical trial partner to Every Cure, where we utilize our clinical development and rare disease expertise to identify links between rare diseases and generic drugs.

We also work with KOLs globally, utilize cross-border enrollment, employ DCT/hybrid studies when strategically advantageous, and apply social listening to adapt to a study's unique needs.

## Building Sustainable, Effective Patient Advocacy Group Relationships

We focus on building strategic, mutually beneficial relationships with patient advocacy groups and currently **have a relationship with 115+ groups globally across all therapeutic areas**. Unlike other CRO, our relationships are personal and are underpinned by a "pick up the phone" approach rather than a corporate relationship. These relationships help us build strong patient engagement strategies with indication-specific patient, parent, and caregiver journeys to improve the patient experience, reduce the burden to participate, successfully enroll trials, and help them advance their missions.

## Ongoing Patient Partnerships

- Autoimmune
- Hematology
- Hepatology
- Infectious Diseases
- Metabolic
- Nephrology

- Neurology
- Ophthalmology
- Pediatric
- Respiratory
- Rheumatology
- General Rare

Check out additional information on our rare disease services or get in touch with us to discuss your rare disease trial. [→](#)



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Worldwide Clinical Trials is founded on an unwavering commitment that combines scientific rigor with personalized attention.

For more information on how we can partner with you to support your clinical trial, please [contact us](#).