

UNIQUE GLOBAL EXPERTISE PROVIDES END-TO-END SUPPORT FOR RARE AND ORPHAN DISEASE THERAPIES

Rare diseases create novel regulatory and operational challenges. Precision for Medicine, Oncology and Rare Disease can help you meet them. We combine highly specialized regulatory and clinical development expert in rare diseases with integrated biomarker and specialty labs—all with a global footprint that enables us to meet the challenges of working with small, geographically dispersed patient populations.

We design studies for small sample sizes and protocols that incorporate the patient perspective to minimize patient burden and increase retention.

Rare disease starts with the patient, so do we. We work with patient advocacy groups and key opinion leaders. Apply thoughtful and creative statistical design for small populations. Provide support to sites and care for patients throughout the trial to maximize recruitment and retention.

Rare disease trials target small patient populations that are spread over a wide geography. That can cause regulatory hurdles, operational challenges, and cultural barriers. Our specialized expertise and global footprint make us uniquely qualified to help you address issues before they become problems, so you can achieve your clinical development goals rapidly.

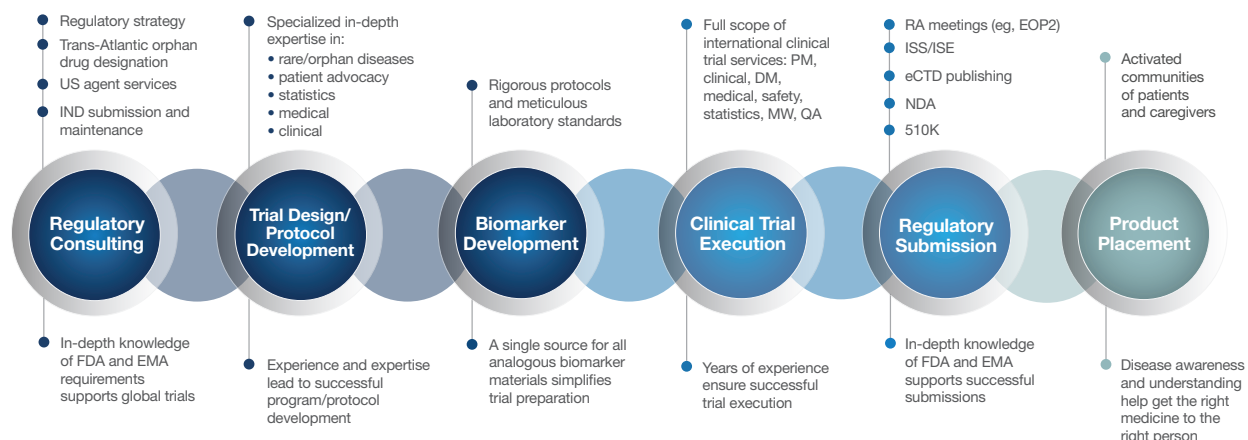
EXPERIENCE
WITH
OVER **40**
RARE
DISEASES

NEARLY
200
ORPHAN
PROJECTS



Our global footprint ensures support around the world.

Delivering treatments for rare and orphan diseases requires specialized expertise. We provide it end-to-end.



DM, data management; eCTD, electronic common technical document; EOP2, end of phase 2; IND, investigational new drug; ISE, integrated summary of effectiveness; ISS, integrated summary of safety; MW, medical writing; NDA, new drug application; PM, project management; QA, quality assurance; RA, regulatory authority.

Rare and orphan disease solutions

- **Patient Recruitment and Retention:** Natural history studies, social media, specialized databases, retention strategies
- **Project Feasibility:** Protocol review, competitive analysis, and site assessment
- **Project Management:** Development of project operational strategy; coordination and oversight of study activities, including study team, timeline, deliverables, reporting, budget management, risk assessment and mitigation, and vendor management
- **Clinical Operations:** Investigator selection, regulatory document collection and submission, TMF set-up and maintenance, clinical monitoring and site management
- **Data Management:** Database design and validation, data cleaning, EDC
- **Biostatistics:** Protocol review and study design, sample size determination, randomization schedules, SAPs, TLF programming, data analysis, CDISC programming including legacy data conversion, DSMB/DMC establishment and management, ISS/ISE, FDA meeting participation
- **Medical, Regulatory, and Scientific Affairs:** Regulatory strategy and submissions, US agent services, FDA meeting participation, IND maintenance, program/protocol planning, medical monitoring, safety services
- **Specialty Lab Services:** Geneticists, biologists, and statisticians define needs and deliver assays and answers
- **Medical writing:** Protocol writing, IBs, INDs, CSRs, ISS/ISE, MAAs, and NDAs
- **Pricing and Reimbursement:** Evidence-based positioning, trend-off analysis, managed markets insights, closed-loop market access modeling
- **Market education:** Programs and materials for patients, healthcare providers, and caregivers

Proud winner of “Best Orphan Drug CRO”
ROAR Awards for two consecutive years

FOR MORE INFORMATION ABOUT RARE AND ORPHAN DISEASE SOLUTIONS

Please contact us at info-ord@precisionformedicine.com or visit precisionformedicine.com/ord