Worldwide Clinical Trials

Worldwide's Rare Liver Disease Experience

What Makes Rare Liver Trials Difficult:

Rare liver disease trials face a mix of scientific, operational, and regulatory risk. Low and ultra-low prevalence, fragmented standards of care, reliance on surrogate or patient-reported endpoints, invasive procedures, and geographically dispersed patients significantly increase execution complexity and sponsor risk. Failure most often occurs not due to science but due to feasibility, enrollment attrition, or endpoint misalignment.

Worldwide brings deep, hands-on experience across rare and orphan liver indications to help sponsors navigate these challenges and progress programs with confidence.

Broad Team Experience Across Complex Rare Liver Indications

Worldwide has led clinical development programs across a wide spectrum of rare and advanced liver diseases, including:

- Primary biliary cholangitis (PBC)
- Primary sclerosing cholangitis (PSC)
- Autoimmune hepatitis (AIH)
- Decompensated liver disease
- Alcoholic hepatitis
- Acute liver failure (ALF)

This breadth enables informed protocol design, realistic feasibility assessments, and indication-specific operational strategies tailored to heterogeneous and often fragile patient populations.

Regulatory and Scientific Leadership in Orphan Liver Programs

Worldwide supports rare liver programs with integrated regulatory and scientific expertise, including:

- Global trial planning supported by in-country regulatory intelligence
- Early engagement with FDA, EMA, MHRA, and PMDA
- Orphan drug designation and accelerated development pathway strategy
- Pediatric development planning, including PIPs and iPSPs
- Liver safety oversight and DILI causality support led by experienced medical experts

Clinical Trial Technology & Core Services for Rare Liver Studies

- Central and bioanalytical labs (PK/PD, biomarker, pathology)
- Non-invasive imaging: MRI-PDFF, MRE, VCTE/FibroScan, ultrasound elastography
- Blood-based biomarkers: ELF, PRO-C3, NIS4+, FIB-4, APRI, FibroSURE/FibroTest
- Centralized biopsy management with AI-assisted histopathology reads
- Preferred vendor relationships with Echosens, Perspectum, and PathAI

Trusted Network of Hepatology Investigators & KOLs

- Worldwide partners with an established global network of hepatology and gastroenterology investigators with proven experience in rare and cholestatic liver trials.
- Long-standing relationships with key opinion leaders active in EASL, AASLD, and patient advocacy communities enable informed site selection, rapid start-up, and expert protocol input throughout the study lifecycle.

Addressing the Operational Realities of Rare Liver Trials

- Low-prevalence patient identification, including registry-enabled feasibility, pre-screening pathways, and cross-border enrollment strategies
- Endpoint complexity, spanning biochemical, histologic, imaging, and patient-reported outcomes often within accelerated or outcomes-based regulatory frameworks
- Invasive procedures, including liver biopsy logistics, centralized pathology workflows, and consistent histopathologic interpretation
- Symptom-driven endpoints, such as pruritus, fatigue, and quality-of-life scales requiring standardized rater training and monitoring
- Pediatric and adolescent populations, with caregiver engagement, age-appropriate assessments, and long-term follow-up considerations

Global start-up, site activation, and enrollment strategies are designed to minimize delays in indications where timing is critical to successful development.

Amplifying Patient Voice & Engaging Advocacy Programs

- Integrating with patient advocacy organizations for awareness and recruitment
- Ensuring the patient perspective is incorporated
- Using the patient voice to directly guide strategy and early stages of study design
- Putting the patient first drives successful recruitment and retention

Scientific & Medical Expertise to Shape Protocol Design

- 150+ combined years of liver disease clinical research experience
- Consultation on endpoint selection, surrogate endpoints, patient stratification, and enrichment
- Adaptive and innovative trial designs tailored to small, heterogeneous populations
- Long-term outcomes and adjudication committee experience

Rapid, Metrics-Driven Study Start-up for Rare Populations

- Risk-identification strategy to safeguard site activation in low-prevalence indications
- Global start-up coverage: North America, Europe, UK, CIS, MENA, Latin America, APAC
- Cross-border enrollment expertise for dispersed rare liver patient populations

Proven delivery in rare liver:

In a global PSC Phase II program, Worldwide enrolled 110+ patients across 65+ sites in 11 countries and met the enrollment milestone 4.5 months ahead of schedule — supporting positive topline results for the sponsor.

90+	29,500+	210+	100+	6,100+
Countries	Patients Enrolled	Projects	Indications	Site Activations

Global rare disease experience — rare liver indications included

Our rare disease track record speaks to the breadth and depth of experience we bring to complex programs, including rare liver disease— backed by a dedicated team with the specialized knowledge and operational depth to advance your study from design through delivery.

Meet Your Rare Liver Disease Team

Sigrunn Blacoe, PhD
Liver Franchise Lead

- 20+ years' clinical research experience
- Extensive global trial leadership across all major regions
- Strong expertise in hepatology and GI, including PBC (outcomes studies), PSC, MASH, and IBD
- Lead voice on cross-franchise liver strategy

Erin Singh, PhD
Clinical Trial Liaison

- 12+ years spanning R&D, clinical research, and CROs
- Proven leader in site engagement and recruitment strategy
- Global clinical recruitment lead for multiple Phase 3 MASH and PBC trials
- Drives enrollment execution across liver programs

Michael F. Murphy, MD, PhD
Co-founder, Chief Medical and Scientific Officer

- Past President, CRO Division, United Health Group
- Lecturer, Harvard Medical School, Clinical and Translational Research Academy
- Recipient, Clinical Research & Excellence (CARE) Lifetime Achievement Award
- Translational medicine consultation across small molecules, biologics, and ATMPs

Attila Timar-Peregrin, MD, PhD
Therapeutic Advisor

- 25+ years' clinical research experience
- Lead medical expert in 40+ GI and hepatology studies
- Phase 1 to 4 study supervisor, PI, and subject matter expert for CROs and medical centers
- Deep experience in PBC, PSC, MASH, and cholestatic liver disease

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

- 23+ years of global clinical research experience
- Provides significant scientific and strategic design input as an SME for Phase 1-4 rare disease trials and real-world studies
- Develops efficient operational strategies for delivering rare disease research by identifying and reducing the barriers for patients and families to participate

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Your science. Our personalized support. Delivered Worldwide.

To learn more about how Worldwide can support your next rare liver disease trial, contact us at contact@worldwide.com