Worldwide Clinical Trials

Case Study

From FPI to Acquisition: Delivering the Phase Ib Data Package That Established a Potential First-in-Class ADPKD Therapy

A small biotech entrusted Worldwide with its only clinical-stage asset, a program that represented the future of the company. Our team delivered. When the biotech was successfully acquired by a larger company, we ensured the transition did not delay the program.

Challenges

A Novel Mechanism with No Established Operational Precedent

Autosomal dominant polycystic kidney disease (ADPKD) is the most common inherited cause of renal failure, yet treatment options remain severely limited. Our sponsor was developing an investigational microRNA inhibitor with preferential kidney exposure. This would serve as a first-in-class mechanism requiring a Phase Ib design that spans double-blind, multiple ascending dose cohorts and an open-label fixed-dose arm, with genetically confirmed patients (PKD1 and PKD2) across 18 U.S. sites.

Study at a Glance:

Indication:
ADPKD

Phase:
1b

Location:
US, 18 sites

Population:
Individuals 18-70 years old diagnosed with ADPKD

Total Patients Screened:
98

Elevated Screen Failure Risk in a Genetically Defined Population

Enrollment required patients to carry confirmed ADPKD mutations and satisfy stringent eligibility criteria across four distinct cohorts. Early screening activity revealed an elevated failure rate that, left unaddressed, would have prolonged recruitment and enrollment activities, and increased costs before a single subject was dosed.

Central Laboratory Instability at a Critical Juncture

Key efficacy and safety endpoints (e.g., urinary polycystin (PC) as a biomarker of mechanistic response and height-adjusted total kidney volume (htTKV) as a measure of disease progression) depended entirely on a functioning central laboratory. Mid-study, the third-party lab vendor unexpectedly reorganized, which introduced challenges to sample management and data quality.

Mid-Study Change of Ownership

Before the final cohort reached completion, our sponsor was acquired by a large global pharmaceutical company. The incoming organization arrived with substantial cross-functional resources but no historical knowledge of the trial. This effectively transformed Worldwide from a CRO partner into an institutional archivist and continuity anchor during the program's most operationally sensitive phase.

Solutions

Pre-Screening Optimized to Protect Enrollment Efficiency

Our Medical Monitor worked directly with the sponsor team to design a structured pre-screening process, ensuring that only patients meeting core eligibility requirements entered the formal screening funnel. This targeted intervention substantially reduced screen failure rates, preserved enrollment momentum across all four cohorts, and kept the overall study timeline intact. Screen fail rates fell from 48% in the first cohort to ~26% in later cohorts.

Proactive Vendor Management & Sustained Laboratory Oversight

Recognizing the central laboratory as a cornerstone of the primary endpoint data package, Worldwide's vendor management infrastructure maintained close, continuous oversight throughout the trial. When the laboratory vendor changed operations mid-study, the team executed multiple rapid escalations, applied persistent follow-up, and partnered with the sponsor on interventions. These measures ultimately contained the impact on sample integrity and safeguarded the completeness of endpoint data.

A Stable, Dedicated Core Team from Initiation to Close

Personnel continuity was the structural foundation of Worldwide's delivery model for this program. The same Global Project Manager, Clinical Trial Manager, Medical Monitor, statisticians, and Clinical Research Associates who initiated the study in March 2022, guided it through transition, and are in the final stages of transferring completed study data. Doing so preserved institutional knowledge, sustained the close collaborative culture the sponsor depended on, and enabled disciplined critical-path reviews at each pivotal milestone, spanning study start-up, interim analysis, and database lock.

Structured Transition Management Through Acquisition

When the acquisition closed, Worldwide became the single thread of continuity for a program the incoming organization had not been part of initiating. Our leadership closely aligned with the acquirer's designated transition lead through regular one-on-one contact and joint cross-functional meetings spanning statistics, data management, and the trial master file. By prioritizing the relationship, honoring appropriate firewalls, and positioning our team as the expert record of the program's history, Worldwide enabled the incoming organization to inherit a well-documented, compliant study without disruption to the final deliverable.

Results

Enrollment. The study enrolled 4 cohorts, screened 98 patients across 18 U.S. sites, randomized 68 patients, and brought screen failure rates under control through a pre-screening method implemented early in the program. Importantly, these efforts also resulted in the study avoiding excessive costs, bringing the progress back in line with the initial predictions and forecasting.

Data Integrity. Despite mid-study disruptions to the central laboratory, diligent vendor oversight and client partnership safeguarded the quality of critical biomarker endpoint data, ensuring a submission-ready package and on-time database lock.

Acquisition. Phase Ib data demonstrated promising clinical efficacy and safety, with consistent impact on urinary polycystin and htTKV outcomes. The strength of that data attracted the attention of a major global pharmaceutical company, which acquired our sponsor — a transaction underpinned by the trial results our team helped deliver.

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To learn more about our extensive experience in rare disease clinical research and how we can partner with you, contact us today.