Worldwide Clinical Trials

Case Study

Achieving Market Approval in a Phase III Breakthrough Therapy for Lupus Nephritis

When a sponsor partnered with Worldwide Clinical Trials (Worldwide) for their oral therapy for lupus nephritis (LN), there were no FDA-approved treatments, only medications to control inflammation. With recruitment hurdles and a lack of regulatory guidance, Worldwide knew this pivotal Phase III study demanded an expert strategy and tailored solutions.

Study Challenges

01 Patient Recruitment

Often, patients at sites with significant experience with LN trials are already participating in a clinical trial. This study required sites familiar with LN clinical trials with eligible patients not already involved in a different LN study.

02 Unpredictable Medical Events

In this study, strict protocol adherence was challenging because the investigational product (IP) needed to be administered at the start of the proteinuria flare. As a result, patients needed to be able to recognize symptoms of the flare indicating when to visit the site.

"Patients don't feel immediately bad when they're in a nephritic flare. If they flared in between visits, we would miss an opportunity to administer treatment."

Ingrid van Rompaey, PhD
Executive Director, Project Management, Rare Disease

03 Unexpected Natural Disasters

In one month, two natural disasters brought devastation to many individuals involved in the study and threatened its progress. In September 2017, Hurricane Irma blew through the Dominican Republic and Florida, causing the temporary closure of several investigational sites. Soon afterward, an earthquake destroyed one of our sites in Mexico and impacted operations in 16 more sites.

Study Facts

27 Countries

240 Sites

358 Patients Enrolled

Primary Outcome

To determine the number of subjects showing renal response at week 52

Primary Outcome Criteria

- UPCR of ≤0.5 mg/mg
- eGFR ≥60 mL/min/1.73 m2 or no confirmed decrease from baseline in eGFR of >20%
- Received no rescue medication for LN
- Did not receive more than 10 mg prednisone for ≥3 consecutive days or for ≥7 days in total during weeks 44 through 52 prior to assessment

Worldwide's Solutions

1

Strategic Site Deployment

To determine the study's sites, Worldwide shared Phase II trial data with LN investigators, generating interest among sites unaware of the study. As a result, we activated several of these sites with LN expertise that were close to patients for quick access. We provided training and tools to ensure recruitment, screening, enrollment, consent, and assessment practices were carried out correctly.

2

High-Quality Patient Education

Worldwide developed a program to enable patients to self-monitor for proteinuria flares so they would know when to proceed to the investigative site for further evaluation and potential IP administration. Our team followed up closely with sites to ensure protocol adherence by clinicians and patients.

3

Strong Trust Development

Taking a solutions-oriented approach, Worldwide's project team cultivated an atmosphere of trust with the sponsor. When two natural disasters threatened our progress, Worldwide acted quickly to activate new sites and implement shorter sponsor review turnaround cycles, further strengthening our relationship with the sponsor.

4

Tailored Therapeutic Area Expertise

Our lupus-experienced members of the project team, backed by Worldwide's deep expertise in immune-mediated inflammatory disorders (IMID) and proven history in orphan disease studies, were key factors contributing to the study's success. Throughout the study, Worldwide remained committed to the sponsor's success, providing tailored therapeutic expertise from team selection to study close.

Outcomes

- By deploying the right sites, our medical experts were able to **support clinicians in the evaluation and treatment of patients** presenting with nephritic flares.
- As a result of strategic site selection and effective staff and patient education, **the study enrolled ahead of schedule.**
- Our quick pivots enabled us and the sponsor to **achieve database lock on time without compromising data quality**.
- Worldwide and the sponsor **built bridges with investigators and patient advocacy groups**, who in turn, supported the study as we moved from regulatory approval to commercialization

Despite the study's challenges, the therapeutic received FDA approval in 2021 to treat adult patients with active LN.

Worldwide Clinical Trials

Let Worldwide Support Your Next Rare Disease Study

Utilizing our deep medical and scientific experience in IMID and rare disease and our relationships with clinical sites in over 60 countries, Worldwide is a top-performing CRO for lupus nephritis studies. **Contact us** today to learn more about how we can support your drug development program.

Last Update: 11132024