Worldwide Clinical Trials

Case Study

Early Phase Transition Study In A Progressive Rare Disease: A Personal Approach

A biotech company needed a CRO to take over a Phase I single center, inpatient study for a progressive rare disease for a drug with an unknown safety profile. To run a safety study in patients with this disease, Worldwide Clinical Trials needed to develop a thoughtful and strategic plan from recruitment through participation.

A Transition Study

Worldwide oversaw the sponsor's preceding Phase I study for healthy volunteers and had the infrastructure in place to accommodate a similar study in patients.

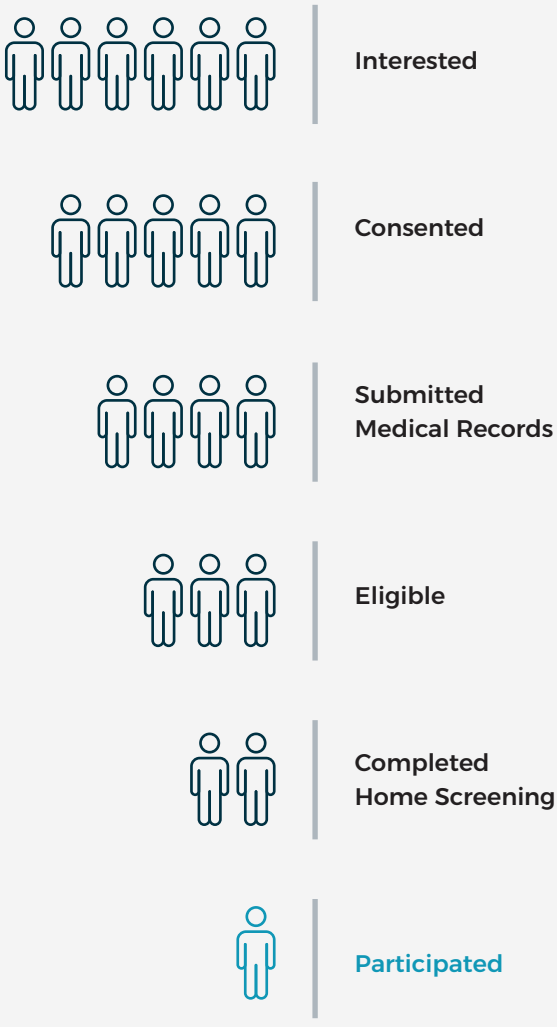
The Challenge

The disorder causes progressive muscle weakness, meaning patients required extra safety considerations and travel accommodations to participate. Because of the health of the patients and the complexity of the therapy, they needed a facility where the study team could monitor patients around the clock. Patients also had to be willing and physically able to travel to San Antonio, Texas, for three weeks for participation.

We came together as one team, through mutual respect of each other's experience and recognition that each team member was making a critical contribution to a study that would make a huge difference — either by bringing to market a new therapy for an ultimately fatal condition or through the lessons we collectively learned by undertaking the challenge."

Derek Ansel, MS, CCRA
Vice President, Therapeutic Strategy Lead, Rare Disease
Worldwide Clinical Trials

Screening Process



The Worldwide Solution

Worldwide implemented multiple strategies to help the sponsor meet its goals, including:

Direct-to-Patient Recruitment

The sponsor utilized a targeted outreach campaign featuring direct contact information for Worldwide's Patient Advocates. Advertisements were featured in industry-specific publications, and clinicaltrials.gov shared the study with potential participants.

01

Hands-on Pre-Screening

Worldwide Patient Advocates established strict patient inclusion and exclusion criteria to identify patients with the lowest risk in participation. Patient Advocates spoke directly with interested patients and families to explain the trial and consented patients to pre-screening. When needed, our Advocates held conversations with families and caregivers regarding follow-up and study experience. Advocates also developed a custom tracker, logging where each patient was in the pre-screening process and tracking screen fail and reasons for drop out.

02

Leveraged Elements of Decentralized Clinical Trials

Using telehealth and similar technologies, the team remotely consented patients to pre-screening, scheduled genetic testing, reviewed medical records, and kept patients informed while patients remained comfortably at home. A home health nursing partner completed additional pre-screening activities at the patient's home. Worldwide also utilized this partner to arrange travel to San Antonio for participants and caregivers.

03

Inpatient Unit

Using our our Clinical Pharmacology Unit (CPU), we provided inpatient care to patients for the duration of the trial, with 24/7 monitoring. The facility was adjusted to fit and accommodate the unique needs of this patient population.

04

Patient Support

Worldwide appointed a patient experience manager who helped patients feel comfortable and accommodated their individual needs during the trial. This approach was well-received by patients, who felt their well-being was cared for and supported.

05

The Results

The outreach campaign far exceeded expectations, bringing in 12x the needed participants for consideration.

After an extensive pre-screening process led by Worldwide's Patient Advocates, the participating patients enrolled.

The study successfully produced the data needed to progress into Phase II trials.

In addition to strong data, the study left a positive impression with patients. According to Worldwide's follow up, patients felt they were thoroughly educated, informed, and supported throughout the duration of the study. Some participants have reported they remain in contact with other study participants.

Why Worldwide?

As a nimble, proactive rare disease-focused CRO, Worldwide presented a solution that met the sponsor's needs and the tight timeline.

Worldwide developed a custom SOP that included patient management, recruitment, cross-border enrollment, and clinical trial management.

With 200 beds and flexible procedure space, our CPU had ample space to keep patients comfortable. Co-located with a GLP bioanalytical lab in Austin, Texas, the study team had easy, rapid access to PK testing and reporting and sample analysis.

Our hands-on approach and dedicated outreach contributed to this study's success. The sponsor met its recruitment goals and gained a few participants interested in its Phase II study.

Not many CROs have the facilities, the recruitment muscle, and the rare disease expertise to execute a Phase I study in patients who have the targeted disease. Worldwide has the talent and the infrastructure to execute these studies in both healthy volunteers and special patient populations on time—always with safety at the forefront.

For more information on running Phase I trials in patient populations, check out our white paper, "**A Novel Approach to Early Phase Studies for Advanced Therapeutics: Screening & Recruiting Rare Disease Participants.**"

Worldwide
Clinical Trials

Worldwide Clinical Trials (Worldwide) is a leading full-service global contract research organization (CRO) that works in partnership with biotechnology and pharmaceutical companies to create customized solutions that advance new medications – from discovery to reality.

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

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