PCM TRIALS
QUALITY MOBILE RESEARCH

PCM Trials Improves Retention in a Pompe Disease Clinical Trial

BACKGROUND

Pompe disease (also called Glycogen Storage Disease type II) is an autosomal recessive liposomal storage disorder caused by pathogenic variations in the acid alpha-glucosidase (GAA) gene. The GAA gene contains the information for production and function of the protein GAA. The genetic variation(s) in the GAA gene cause a shortage in GAA (an enzyme) affecting glycogen processing.

Glycogen, a complex sugar, cannot be degraded to a simple sugar like glucose. This causes glycogen to accumulate in most tissues, but primarily in skeletal muscle, smooth muscle and cardiac muscle, where it causes damage to tissue structure and function. Close to 500 different GAA gene variations have been identified in families with this disorder.

Researchers have described three types of Pompe disease which differ in severity and the age at which they appear. These types are known as classic infantile onset, non-classic infantile onset and late-onset. This study focused on late-onset Pompe disease, a less severe form, with onset in late childhood, adolescence or adulthood. Late onset manifestations typically include difficulties in ambulation and reduced respiratory function.

SITUATION

This Phase 3, adult participants, clinical trial was focused on late-onset Pompe disease and required a seven-hour intravenous infusion every week. Due to the duration of the weekly treatment and the relatively mild symptoms experienced by the participants, the study found it difficult to enroll and retain participants. The rarity of this disease combined with the travel distance required to the site made the study impractical to execute without a home visit component.

CLINICAL TRIAL'S REQUIREMENTS FOR HOME VISITS

A blinded study design required that two nurses visited the participant's location, whether it was in the home, in the office or while the participant was on a vacation. An unblinded nurse prepared the infusion and a second blinded nurse administered it.

PCM Trials was selected as the mobile visit provider for this clinical trial due to its experience with administering in-home infusions and with rare diseases[1]. Additionally, the PCM Trials nurses were direct employees and Certified Mobile Research Nurses (CMRNs), increasing the quality of care provided to the participant and improving quality of data reported to the site.

The PCM Trials project management team, via their Central Pharmacy, was responsible for delivering the IP to the remote location. The PCM Trials project management team coordinated delivery of the IP with the arrival of the CMRN team. PCM Trials separates the logistical responsibilities from the clinical responsibilities, allowing the CMRNs to focus on the subject and the clinical visit objectives.

[1]Rare diseases represent over 30% of the protocols supported by PCM Trials

THERAPEUTIC AREA
Genetic Disease

DIAGNOSIS
Pompe Disease
Late-Onset

CLINICAL TRIAL
Double-blind,
Randomized
Phase 3 Study

DURATION OF TREATMENT
52 Weeks

EVALUATION CRITERIA
6-Minute
Walk Distance

IP DELIVERY
Intravenous & Oral

COUNTRIES
20

SITES
73

PARTICIPANTS
150

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The PCM Trials project management team reviews all source documents from the CMRN after the completed visit. The project manager ensures that the source document has been filled out properly and follows ALCOA-C guidelines. As a result of the 100% inspection of the source documents by the PCM Trials project manager, the home visit portion of the study maintains a high degree of data integrity which minimizes the chances of delays to the study.

A PARTICIPANT'S EXPERIENCE

A college student participated in this clinical trial during the participant's undergraduate studies. There were two CMRN teams assigned to this participant depending on whether the student is away at college or at the family home during breaks.

The CMRN teams were comprised of PCM Trials employees. There was consistency in the administration of the IP, allowing the CMRN team to develop a close rapport with the participant. This rapport boosted the participant's overall adherence to the protocol and increased the participant's willingness to continue with the trial.

This student is now doing post-graduate work. Without the deployment of these home visits, the student would have had to put the degree on hold or not participate in the clinical trial.

ABOUT PCM TRIALS

PCM Trials is the only company that trains, certifies, tests and manages our own Certified Mobile Research Nurses (CMRNs) to give you unmatched quality clinical trial in-home visits. Over the last decade, we have helped numerous sponsors and CROs conduct alternate site research across more than 300 protocols in a wide range of therapeutic areas around the world.

THE PCM TRIALS DIFFERENCE: OUR CMRNS AND CONSISTENT, STANDARDIZED PROCEDURES

PCM Trials is the only mobile clinical trial research company that employs Certified Mobile Research Nurses (CMRNs). Since we do not solely rely on third-party home health care agencies, we train and test our CMRNs in Good Clinical Practice (GCP) principles, International Air Transport Association (IATA) standards and protocol procedures. They understand the complex needs of clinical trials and follow one set of Standard Operating Procedures (SOPs).

By making certain our CMRNs are well-qualified and having complete control over processes, we reduce risk of protocol deviations and maximize data quality and safety.

BENEFITS OF IN-HOME CLINICAL TRIAL VISITS

Our CMRNs make hundreds of in-home visits each month. By offering these convenient and cost-effective services, your company can:

- Increase participants' willingness to participate by minimizing the potential stresses associated with site visits
- Help participants feel more engaged in the study
- Improve adherence to your protocol
- Reduce drop-out and 'lost to follow up' rates

For sponsors and CROs, such improvements in participant recruitment, retention, and compliance mean your clinical trials can be completed faster, which may result in lower costs. To learn more about how PCM Trials services can help your company, contact **info@PCMTrials.com** or call **888.628.9707**.

PCM Trials' mobile nursing services are available throughout the U.S., Puerto Rico and internationally.

For more information on PCM Trials, please call us at **1.888.628.9707** or **info@PCMTrials.com**.

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