PCM TRIALS
QUALITY MOBILE RESEARCH

Home Visit Nurses Deliver Complete Trial Data Despite A Challenging Population, A Pandemic And Severe Weather

BACKGROUND

Rett syndrome, a rare progressively degenerative neurological disorder that occurs mostly in girls, is caused by mutations in the X-linked gene methyl-CpG-binding protein 2 (MECP2), a pervasive transcriptional regulator. At a very young age—often between 6 and 18 months of age—girls with classic Rett syndrome start developing severe problems with language and communication, learning, coordination and other brain functions, often accompanied by seizures. Rett syndrome is estimated to affect 6,000 – 9,000 patients in the U.S. alone.

SITUATION

This Phase 3 clinical trial focused on a fragile female pediatric population diagnosed with Rett syndrome. This particular patient population can be challenging due to the patients' mobility, inability to communicate, and frequent seizures.

The trial spanned a number of years, including the time designated as the peak of the COVID-19 pandemic. The trial started as a double-blinded study, and then participants from nearly 40 states were grandfathered into the open-label study and, eventually, the long-term extension study.

Complex visits scheduled every 2-4 weeks included vitals analysis, ECGs, and urinalysis, as well as complex PD/PK sampling. With COVID-19 threatening the viability of the study by closing numerous sites and making it difficult for participants to reach those sites that remained open, the sponsor amended the study protocols to allow the increased use of mobile research nurses and include additional safety visit types.

APPROACH IN OVERCOMING CHALLENGES

From the outset, this study was designed to utilize a hybrid approach whereby mobile nurses worked closely with sites to ensure consistency of data and compliance with protocols. Initially limited to IP accountability, the role of PCM Trials' nurses quickly expanded in response to challenges created by COVID-19.

THERAPEUTIC AREA
Rare Neurological, Progressively Degenerative Diseases

INDICATION
Rett Syndrome

TRIAL DESIGN
Double Blind, Randomized, Phase 3 Trial

DURATION
4+ Years

COUNTRIES
United States

SITES
20

PATIENTS
150+

HOME VISITS COMPLETED
750+ Double Blinded, 1,200+ Open Label and Long-Term Extension

With so many sites shut down or otherwise inaccessible, PCM Trials certified mobile research nurses quickly assumed additional responsibility for safety visits. Due to PCM's ability to quickly ramp up its operations and ensure the timely delivery of accurate data in-window, many sites (even after they had reopened) asked PCM nurses to continue their home visits with additional requests to also handle blood draws and ECGs.

Even during the early stages of the trial, it quickly became apparent that home visits by specially trained nurses served to significantly ease the burden on participant families by eliminating the need for them to travel to sites. This was significant since many of the participants lived in remote, hard-to-reach, underserved areas—as far as four hours from the closest site. Equally important was the flexibility of the PCM mobile nurses to work around school and caregiver schedules. Such flexibility and convenience played a key role in being able to recruit and retain participants despite the lengthy duration of the study.

To ensure compliance with the trial's protocol, PCM Trials provided robust, protocol-specific training to its nurses, including refresher training modules focused on special processes required for the added safety visits. PCM hired only RNs or pediatric nurses with comparable advanced nursing degrees to ensure they possessed the necessary skills to handle ECGs and blood draws with highly volatile patients who were prone to sudden mood changes and seizures.

The COVID-19 pandemic as well as a severe winter storm in Texas compounded logistical and supply chain challenges. Working collaboratively and proactively with the various sites and its extensive network of vendors, PCM Trials was able to develop creative backup plans and manage unexpected delays to ensure on-time delivery of the IP to patients' homes and the safe delivery of frozen samples packed in dry ice.

RESULTS

PCM Trials mobile research nurses were able to successfully collect data and deliver samples in a timely manner, despite the challenges created by a deadly pandemic and dangerous weather. The trial also had to successfully recruit and retain enough participants to validate the results. With the added convenience and flexibility of mobile research nurses making home visits, the trial was able to over-enroll participants.

PCM Trials' mobile nurses played a critical role in ensuring patient compliance with the trial protocol. Not only did PCM nurses have responsibility for caregiver training and support, but the low turnover rate in nurses ensured consistency of data collection and helped build rapport with trial participants and their families. Nearly 60% of the original PCM Trials nurses completed the trial. Similarly, more than 50% of the enrolled patients remained in the trial throughout all three of its stages.

To learn more about the benefits of incorporating home nursing visits into your next clinical trial, contact PCM Trials at 866.631.9985 or info@PCMTrials.com, or submit an RFP today.