CASE STUDY PRESENTED BY PREMIER RESEARCH

BACK ON TRACK: DATA RESCUE ON A PEDIATRIC RARE DISEASE TRIAL

Imagine rebuilding the engine in an ambulance carrying a critically ill patient to the hospital. Got it? Then you have a pretty good idea what it took to put this pediatric rare disease trial back on track.

A pharma company developing a drug to treat urea cycle disorder struggled with a CRO that was not effectively managing the study data. As trouble mounted, the CRO quit, stranding the project at a critical point in the development cycle.

Longstanding relationship

On the strength of a longstanding relationship, the sponsor chose Premier Research to take over the data management and statistics portion of the trial, which was evaluating a drug used in adults for pediatric use. Early in the handoff process, the deficiencies in the trial's electronic data capture system became more apparent. The incumbent system was deemed too cumbersome and was unable to capture all of the required endpoints.

That prompted the sponsor to switch technology platforms, moving the trial to Medidata Rave just as a full data analysis for the sNDA submission was fast approaching its due date.

Aggressive timing

Crossing the language barrier between the legacy system and Rave was a major hurdle, and being fluent in both, we worked fast to create translation documents, rewrite programmed edit checks, and create new edit checks to overcome the old system's limitations. The schedule was very aggressive: less than eight weeks from the initial discussion to the switchover weekend, when the study went live in Rave.

The handoff was a success, and the data analysis was delivered on schedule. The trial remains in progress, still enrolling and conducting patient follow-up.

BIOMETRICS

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Crossing the Language Barrier to Rescue a Pediatric Rare Disease Trial

Study Description:
Phase IV PMR trial of a drug to treat urea cycle disorder in patients under two years of age

Therapeutic Area:
Pediatric rare disease

Services:
Data management and biostatistics

Geographic Scope:
17 sites in the United States

Patient Population:
Pediatrics

Enrolled, randomized, and treated:
20, trial is still enrolling

Length of Enrollment Period:
23 weeks

Outcome:
Transition to Medidata Rave was completed on schedule and in time to deliver a full interim data analysis. The trial is ongoing, on track, and continues to enroll patients.

premier research
IT'S WHAT WE DO. BEST.™

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