Case Study:

ACHIEVING SUCCESS IN A GLOBAL FRONTOTEMPORAL DEMENTIA (FTD) TRIAL THROUGH A COLLABORATIVE 'ONE-TEAM' APPROACH

A Multi-Center, Double-Blind, Placebo-Controlled Trial for Amyotrophic Lateral Sclerosis (ALS) and/or Frontotemporal Dementia (FTD)

8 COUNTRIES | 3 REGIONS

PHASE I/II STUDY

43 PATIENTS

17 SITES

OPEN LABEL TO INDEX PHASE I/II STUDY

35 PATIENTS

7 SITES

CHALLENGES

- Volume of complex procedures with patient population experiencing disease progression and decreasing irritability threshold
- CSF sample tracking and processing at up to three specialty/external labs across three geographical regions
    - Pre-lumbar puncture platelet/prothrombin-time result turnaround time
    - Less frequent of batch runs of specialty biomarker assays
    - Sample processing and aliquot management of local and central samples at ambient, refrigerated, frozen conditions
    - Necessary equipment (e.g. refrigerated centrifuge at multiple RPM levels)
- Adherence to PK series collection timepoints, as out of window collections tend to cascade
- Fluctuating ad hoc dose escalation and data safety monitoring board meetings due to dynamic design requiring agility to recruit expanded cohorts, and ensure data readiness for rolling committee reviews

THE SOLUTIONS

- Proactively planned cross-departmental coordination (lumbar puncture team, local lab, CRCs, functional assessments raters, etc.) to facilitate seamless visit experience with backup plans built into visit itineraries
- Pre-visit huddle with CRA, primary CRC, and Investigators to review logistical plan for milestone timepoints critical to endpoints and contributing department team leads to ensure reminder messaging to extended site teams and patient/family
    - Ongoing communication between CRA, CTM, and site with well-defined check points throughout dosing/LP day through completion of all split shipments to central lab
    - Site contact check points clearly defined in the CRA Monitoring Plan supporting consistent documentation
    - Close collaboration between Medical Monitors, Principal Investigators, and key sub-Investigators during enrollment
    - Involvement of lab manager or lead lab technician from SIV through final lab shipment
    - Patient schedule details shared with central lab team during weekly routine calls to evaluate risk as a team
- To enhance shipment tracking oversight, sites provided copies of airway bills to CRA and central lab for critical sample shipments contributing to upcoming dose escalation/DSMB review meetings to permit priority intake by central lab and prompt shipment to external lab
- Detailed lab manual instruction on aliquot packing, specific to shipper condition, to maximize sample stability
- Developed patient-specific shipment plans during holidays, accounting for local holidays impacting courier services
- For PK series and complex procedures, conducted extensive site training and shared knowledge and lessons learned with Investigators periodically to maintain expertise in protocol procedures
    - Split shipment approach to minimize risk of courier delays compromising all PK/CSF samples for a given visit
    - Reviewed lab requisition forms and tips at each site teleconference to minimize queries
- Frequent review of lab portal to ensure accuracy of sample receipt report and site supply/kit inventory report

RESULTS

The close partnership between the Sponsor, Medpace and the sites through a 'one-team' approach led to:

- Rapid enrollment of the study
- The last patient being enrolled ahead of schedule
- Achieving periodic DSMB data cuts
- Parallel final database lock on time for both studies

FULL-SERVICE CLINICAL DEVELOPMENT

Medpace is a scientifically-driven, global, full-service clinical contract research organization (CRO) providing Phase I-IV clinical development services to the biotechnology, pharmaceutical and medical device industries. Medpace's mission is to accelerate the global development of safe and effective medical therapeutics through its high-science and disciplined operating approach that leverages local regulatory and deep therapeutic expertise across all major areas.

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