White paper

Decentralized clinical trials

New clinical frontiers in neuroscience

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The COVID-19 pandemic has highlighted the need for evolution and flexibility in clinical research, particularly in the area of neuroscience. As a result, the industry has turned its attention to hybrid and decentralized clinical trials (DCTs) to protect current research endeavors while incorporating agile options in future protocols.

Addressing challenges in neuroscience with technology

Over the last year, life science leaders have increasingly acknowledged that neuroscience clinical studies are favorably positioned to shift away from traditional modes of clinical conduct and to increase the use of decentralized clinical trial (DCT) options. Digital components, such as devices, ECOG scoring, remote eConsent, and eDiaries, along with TeleVisits, home health care, and direct-to-patient (DTP) supply and direct-from-patient (DFP) sample collection, are key parts of the equation.

In particular, mobile technologies that track patient movement with a wearable patch are of particular interest for study teams evaluating the digitization of, or remote collection of, endpoints. While the collection of data from wearables is widely performed, the interpretation of the aggregated data can be a challenge for researchers—particularly in the prediction of the various iterations of dementia. Thus, the industry is focusing on employing digital biomarkers as a means to provide clarity and direction that, when combined with patient-reported outcomes (PROs), allow for greater insights.

For some neurodegenerative diseases, there is a gap between the diagnostic criteria that a physician might use and the standardized criteria needed for a clinical trial, which can result in a failure to translate preliminary recruitment into enrollment. For instance, in Alzheimer's disease, enrollment involves the National Institute on Aging–Alzheimer's Association (NIA-AA) research framework released in 2018 and a specific positive Alzheimer's disease biomarker. Most Alzheimer's studies require inclusion criteria of either a PET scan to prove amyloid positivity or cerebro-spinal fluid (CSF) or sampling, which can pose difficult challenges in remote implementation. However, a serum test has been developed to fit the research framework and the regulations with a remote solution used as a gateway prior to proceeding with PET or CSF.[1] In this case, a home healthcare nurse retrieves a blood sample from a patient—lowering traditional clinical barriers of distance to a site. This can be a more efficient and cost-effective alternative to the burden of traveling to a site for a PET scan. While Alzheimer's disease in particular poses additional challenges stemming from its unique cognitive endpoints, individually approaching each protocol and forming fit-for-purpose designs has shown utility.

With neuroscience research, the PPD™ clinical research business of Thermo Fisher looks to lessen the burden at individual sites by equipping them with remote capabilities. As a result, our network has the proven ability to accelerate enrollment for clinicians and sponsors. Without traditional geographic barriers or limitations, patients can be evaluated at any of our sites and remotely recruited where they would fit best, including based on disease prevalence.

Overall, a deep and comprehensive evaluation of the assessments and endpoints required or performed for each study is necessary to make a determination of the proper mix of technology solutions, including the needs of patients within that indication. We can provide guidance, leveraging their deep-rooted experience in product development and medical expertise in the neuroscience space.

Support for patients from the start

Patient recruitment in the neuroscience space demands flexibility and adaptability. This is echoed in new FDA guidelines released in November 2020 aimed at enabling greater diversity in clinical trials through changes to eligibility criteria, enrollment practice, and trial design. Study teams must be hyperconscious of the needs of minority patient populations, where a decentralized option may be the best route to increased enrollment. We work closely with patient advocacy groups to understand how new research modalities can foster convenience and ease for those living with a neurological indication. We have a number of active initiatives in place to achieve greater diversity, including metric tracking, recruiting diverse principal investigators, identifying sites in areas that better reflect the disease population, and decentralized methodologies that make it easier for all to participate in clinical studies.

On top of that is the patient's need for dedicated support and resources. We offer a patient concierge service to coordinate everything from travel and logistics to patient-centered care within study protocols. The patient concierge is assigned to support each patient and caregiver for the duration of the study, helping to navigate through the complexity of a clinical trial. Our concierge frequently checks in with participants to answer questions, provide reminders, and serve as a source of motivation. This type of personalized outreach reduces drop-outs, which can make it easier to achieve study milestones and can enable faster decisions.

Tackling complex and diverse neuroscience studies

A patient-centered approach to neurology trials has evolved with remote decentralized monitoring being made possible by DTP and DFP shipments, mobile technology, digital wearable solutions, telemedicine, home health visits, and alternative site strategies (including mobile units and virtual sites). We stand at the forefront of these solutions.

Our neuroscience unit has completed over 310 trials in the past five years, successfully reaching over 85,000 patients. The unit is divided into primary therapeutic pillars: neuroinflammatory disorders, rare disease, ophthalmology, neurodegenerative disorders, movement disorders and pain, psychiatry, neurobehavioral, sleep disorders, and epilepsy. In particular, the rare disease and ophthalmology pillars have seen significant growth, with considerable expansion into neuroinflammatory disease. To bolster these pillars, we have dedicated centers of excellence, including rare diseases, pediatric trials, and movement disorders. Other areas of development include Alzheimer's disease and dementia — particularly frontal temporal lobe dementia, as well as myasthenia gravis and amyotrophic lateral sclerosis. All of these neurologic diseases have required crossing therapeutic pillars, including into the rare neurodegenerative and neuroinflammatory areas.

Our neurodegenerative disorders innovation group comprises members from across the enterprise, creating a multifaceted resource for sponsors. The industry's leading biopharma and biotech organizations look to us for study protocol support and analysis across multiple pillars of clinical excellence. Our neurology teams work cross-functionally to develop the best protocol strategy and provide technology-agnostic recommendations that best suit the needs of study stakeholders — ultimately broadening the horizon for neuroscience patients.

References

1. C2N Diagnostics Receives Breakthrough Device Designation from U.S. FDA for Blood Test to Screen for Alzheimer's Disease Risk. C2N Diagnostics. 28 Jan. 2019.

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