

Checklist

Strategic Planning for Neurodegeneration Studies: A Checklist to Navigate Study Complexity

Neurodegeneration studies are renowned for their complexity and for requiring dedicated research staff. It is often necessary for the investigator's budget to exceed fair market value, as quality sites can choose between studies, and may direct their efforts to similar studies with more appropriate compensation for their efforts. This is an important aspect of study planning, as it is a significant factor in the finances needed to run the study and directly affects enrollment and overall study timelines. Below, we provide critical questions that sponsors must address to optimize trial outcomes from the start.

1

Thorough Evaluation of Site Infrastructure

Does your program:

Have access to the right population of participants?

Have access to specialist testing facilities such as volumetric MRI and/or PET Imaging?

Plan for tracer availability to sites (manufacturing sites dictate where clinical sites can be placed)?

Have availability of qualified raters?

Limit direct competition?

2

Participant Selection

Do you:

Have access to the appropriate pool of participants? This needs to be discussed and vetted prior to site selection, and it includes not only "database reviews" but also discussions about how the site plans to attract participants after they have gone through their database.

Have tiered screening approaches to ensure that high-cost assessments are only conducted for potential participants who do not fail screening for any other reason?

Prepare an evaluation of specific population requirements, whether this is a biomarker signature or a clinical subtype? Many of the current genetic or biomarker population-enrichment criteria are not used in clinical practice and are not communicated to sites prior to screening. This needs to be factored into study planning.

3

Clinical Outcomes Assessment Scales

Study sponsors are increasingly looking to expand their geographic footprint to avoid direct competition, particularly with highly enriched populations such as those with genotypic variants.

Have you ensured that validated translations of the required COA scales are available and supported?

Are you sure that the relevant biomarkers or PET traces, when needed, will be available in the region and will pass regulatory scrutiny for use?

4

Participant/Lived Experience

Scientific endeavors should be balanced with participants' perceptions of study attractiveness, which affects recruitment and retention.

Have you prepared a judicious selection of study procedures, assessments, and endpoints, considering invasiveness and impact on the duration of study visits?

Does your protocol provide support services to help participants overcome daily contentions that may otherwise prevent their continued participation?

Have you enabled continued access to investigational products to minimize incomplete data?

5

Biomarkers:

Are you prepared for the varying levels of validation required for fluid-, tissue-, or imaging-based biomarkers, depending on the context of use?

Do you have the requisite IVDR compliance for biomarkers implicated in clinical decision-making?

Are you using biomarker testing kits that conform to E.U. safety, health, and environmental protection standards, as indicated by the CE marking?

See our [white paper](#) for more information about employing biomarkers in neurodegenerative trials.

6

Time to Implement Technology

Does your timeline account for vendor services, including technology such as digital study tools, which are on the critical path of study start-up?

Do you have a trusted vendor coordinator who offers close attention to the details of user requirements specification, user acceptance testing, validation, and implementation?

Can you quickly submit studies to global regulatory bodies outside of the U.S.? Regulatory bodies expect screenshots of these eCOA assessments, which may delay the timeline.

7

Study Partners

Across neurodegenerative research, the study partner, who is often a caregiver or family member, plays a pivotal role in enabling research. From aiding the study participation visit to accompanying the participant to each site visit, the study partner is forming a long-term commitment to the study.

Do you consider the full picture of costs on the study partner, beyond financial, to include support for emotional, health, and societal consequences?

Are your participant instructions clear enough that study partners can become experts, savvy with study technology, IP handling, and overall compliance with the study schedule?

Do you include the often-overlooked caregiver burden scale in later-stage studies to quantify the holistic benefit of an efficacious pharmacotherapy?

We reviewed these concepts in our article about [caregivers in neurodegenerative trials](#).

Worldwide Clinical Trials can manage your global study down to the smallest detail. We bring our client-centered ethos to every partnership, designing service solutions that fit your specific needs. Contact us for more information on our neurodegenerative experience or to talk about your study.

[Contact Us](#)