NeuroNEXT Clinical Study Concept Synopsis

Date:

Title:	
Principal Investigator(s):	**Institution (name and address):**
Phone:	**E-mail address:**
Under which NeuroNEXT-specific PAR do you intend to submit your application: ☐ **NeuroNEXT Clinical Trials (U01) (PAR-11-343)** ☐ **NeuroNEXT Infrastructure Resource Access (X01) (PAR-11-344)** ☐ **NeuroNEXT Small Business Innovation in Clinical Trials (U44) (PAR-11-345)**	
Can the information provided in this form be circulated within the NeuroNEXT Network for purposes of determining feasibility? ☐ **Yes** ☐ **No, please provide a brief synopsis of your proposal that can be circulated within the Network**	

Project Description

Target Disease:
Investigational agent (drug/biologic/device):
Primary aims of the trial:
Secondary aims:
NeuroNEXT is intended for exploratory studies (including biomarker discovery) that will result in go/no-go decisions for a future Phase 3 efficacy trial. In 150 words or less, please state the question that you wish to explore in this trial:
If the proposed study is positive (meets the go decision), please describe what the possible study design for next study (ex. Primary objective, primary outcome) and how it would differ from the currently proposed study:
Briefly describe the scientific rationale for the trial:
Briefly describe relevant pre-clinical and/or clinical evidence used to support this trial-addressing the questions below where applicable (http://grants.nih.gov/grants/guide/notice-files/NOT-NS-11-023.html): **Which animal models were used for the preclinical evaluation?** **Were control animals used during the preclinical evaluations?** **Describe the steps taken to minimize bias during the conduct of the preclinical evaluations.** **Have the preclinical results been independently replicated?**

Is there evidence that the interventional agent reached and engaged the target?

Describe the route/timing of the intervention delivery/dosing.

Describe any clinical data, if any, that supports this study.

Briefly describe the proposed trial design:

Patient selection criteria:

Inclusion Criteria

Exclusion Criteria:

List participating pharmaceutical, biologic or device manufacturing companies (if any):

Is the investigational agent (drug/biologic/device) under an open IND/IDE? Yes ☐ No ☐
If yes, IND/IDE number:_____

If no, will the proposed study be performed under an IND/IDE? Yes ☐ No ☐ Unknown ☐

If yes, has this protocol been submitted to the FDA? Yes ☐ No ☐
Please note our policy requiring documentation from the FDA regarding the status of the protocol you wish to implement: http://grants.nih.gov/grants/guide/notice-files/NOT-NS-11-018.html

If no, has the FDA provided a written exemption from the IND/IDE requirement? Yes ☐ No ☐

Do you or any member of the study group have a financial conflict of interest or hold a patent with the use of the intervention? Yes ☐ No ☐

What specific outcomes would make you determine that the investigational agent/biomarker warranted further study, e.g. a Phase III trial?:_____

What specific outcomes would make you determine that the investigational agent/biomarker did not warrant further study, i.e what would cause a 'no-go' decision?:_____

Have you (or one of the Co-investigators) received past NIH funding for the preliminary work leading to this proposed trial? If so, please list the grant numbers and titles.

Is your institution a NeuroNEXT clinical study site (not required)? Yes ☐ No ☐ If yes;
Have you discussed this proposal with the NeuroNEXT PI at your institution? Yes ☐ No ☐
Can we copy the NeuroNEXT PI from your site on correspondence about this proposal?
Yes ☐ No ☐

Is your institution a CTSA site (not required)? Yes ☐ No ☐
If yes, have you discussed this proposal with your CTSA's protocol development group and/or presented it at a CTSA Brainstorming Session / Studio / Mock Study Session? Yes ☐ No ☐

Are there "other resources" at your institution that you have used in developing this proposal?
Yes ☐ No ☐
If yes, describe: _____

Was this proposal developed in conjunction with a NeuroNEXT Brainstorming Session (not required)?
Yes ☐ No ☐

If this is an X01 and/or U44 application, was this proposal developed in conjunction with the NeuroNEXT Pipeline Development Committee (not required)? Yes ☐ No ☐

Statistical Considerations:

For NeuroNEXT Network funded clinical trials, there are two general approaches that can be taken with respect to the statistical design and analysis activities:

1) For many funded trials, the NeuroNEXT Data Coordinating Center (DCC) performs all statistical design and analysis functions.
2) In other situations, if a Protocol Principal Investigator has previously worked with an external biostatistician, they may be allowed to join the project, under the following conditions:

- The external biostatistician will work collaboratively with the NeuroNEXT DCC.
- The NeuroNEXT DCC statisticians will serve as the unblinded statisticians for the trial
- The external biostatistician will be blinded to safety data and interim analysis results during the course of the trial
- The external biostatistician may only receive raw blinded data or datasets during the course of the trial if and when permitted or required by NINDS and the DCC PI
- The external biostatistician may be included as a blinded participant on the Protocol Steering Committee (PSC) or other relevant NeuroNEXT committees and may serve as a statistical advisor to these committees
- The external biostatistician may participate in the development of the Statistical Analysis Plan, in collaboration with DCC biostatisticians
- The external biostatistician may collaborate with the DCC biostatisticians in the final study analysis (if agreed upon by NINDS and the DCC)

Do you plan to request the use of an external statistician for this protocol? ☐ **Yes: Please provide the name of the external statistician, contact information, and a rationale for the need to involve the external statistician.** ☐ **No**
Please provide a 'guesstimate' of your study sample size to assist with the feasibility assessment. If this proposal moves on to a PWG, the DCC will help further define the sample size. **Proposed number of subjects to be enrolled:** **Describe the statistical basis for the proposed sample size calculation:**
List proposed statistical methods to be used to analyze the primary and secondary aims of the trial:
Additional information (optional) *please list applicable references for your proposal here*: