SMA biomarker study
RFA–NS–12–004
Initial project for NeuroNEXT
Housekeeping issues

- Please mute your phones
- If we disconnect due to technical difficulties, please remain on the line and wait—we will reconnect
- We will not be using the webinar “chat” or “raise your hand” function. All communication will be done through email to Next@ninds.nih.gov
- The slides from this presentation will be posted on the website.
- I will speak for about 10–15 minutes then I will entertain questions.
Key dates

• Letter of Intent (LOI)
  – Submission is optional
  – Due date if you choose to submit LOI is August 1, 2011
• Application Receipt
  – September 1, 2011
• Announcement of study to be funded
  – February 2012
• Study start
  – April–May 2012
NIH/FDA SMA biomarker workshop

- Held May 13, 2011
- Summary of talks along with a set of slides is available at www.ninds.nih.gov/NeuroNEXT
Mandatory aspects of the study

- Must study a putative biomarker in SMA
- Natural history study in SMA 1 patients must be conducted concurrently
  - This \textit{does} need to be included in the applicant’s budget
- Biospecimens must be sent to the NINDS repository at Coriell
  - This \textit{does not} need to be included in the applicant’s budget
How many biomarkers to study?

- Minimum—one (1)
- Maximum—Applicant’s discretion
What sort of biomarkers to study?

- Applicant’s choice may include one or a combination of the following:
  - Prognostic biomarkers
  - Predictive biomarkers
  - Pharmacodynamic biomarkers
- Candidate biomarkers for SMA diagnosis will not be considered.
IND requirement

- If the applicant plans to use an investigational agent for proof of principle, an IND will be required at the time of award.
- If the applicant does not plan to use an investigational agent, no IND will be needed.
NeuroNEXT and this RFA

- The applicant does not have to be affiliated with an institution which has applied to NeuroNEXT in order to respond to this RFA.
- It is necessary to work with the NeuroNEXT infrastructure to conduct the study.
The Data Coordinating Center, Clinical Coordinating Center and the Clinical Sites will all be announced in late September.

Applicants for the SMA Biomarker study should list their proposed collaborators as they normally would in a grant application. Any necessary budget adjustments will be made after the DCC, CCC and Clinical Sites are chosen.
Infrastructure

- NeuroNEXT clinical sites will participate in this study
- NeuroNEXT DCC will be expected to perform the statistics and data management for this study
- NeuroNEXT CCC will collect data from the involved clinical centers
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Where may I find FAQs about this RFA?

- www.ninds.nih.gov/NeuroNEXT
Contact information

- Scientific contact for this study
  - Elizabeth McNeil MD MSc
    - mcneilde@ninds.nih.gov

- Contact for CRADA discussions
  - Laurie Arrants
    - arrantsL@nih.gov

- General NeuroNEXT mailbox
  - NEXT@ninds.nih.gov