

## NeuroNEXT-Presentation at American Epilepsy Society D. Elizabeth McNeil, MD MSc December 8, 2013



# Scope of NeuroNEXT

- Exploratory studies
- Biomarker studies intended to inform trial design
- The Network also accepts proposals for adaptive, seamless Phase II/III designs for diseases with prevalence of <5,000 in US
  - The CCC and DCC will work with PPIs to assist in developing these proposals once approved by NINDS

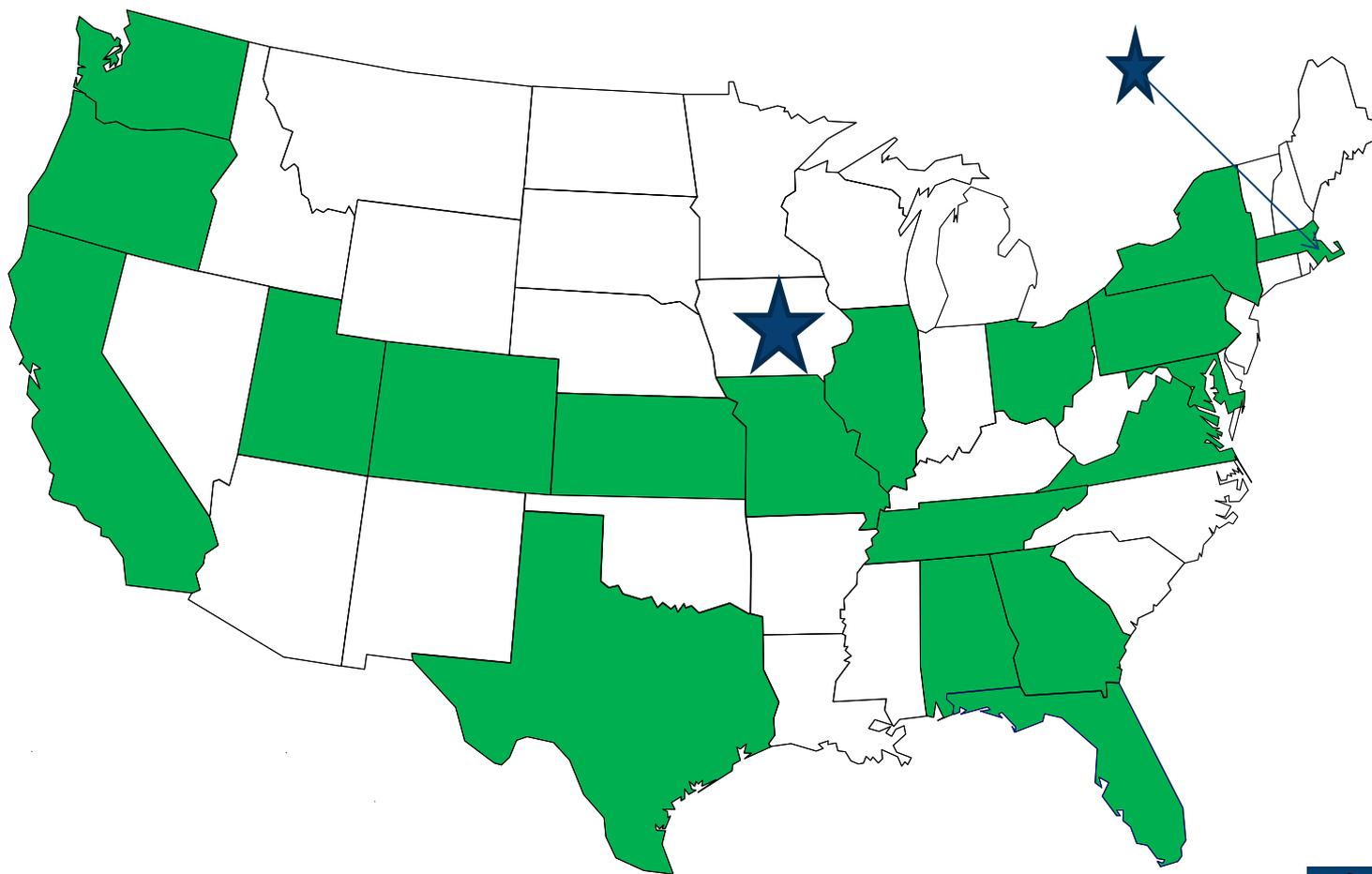
# Coordinating Centers

- Clinical Coordinating Center (CCC)
  - Mass General Hospital
- Data Coordinating Center (DCC)
  - University of Iowa

# Clinical sites

- Albert Einstein College of Medicine- Yeshiva
- Children's of Boston
- Children's National
- Columbia/Cornell
- Emory, Atlanta
- Harvard Partners (MGH/BWH)
- Northwestern University
- Ohio State University
- Oregon Health and Science University
- Swedish Health Services (Seattle)
- SUNY (Buffalo, Downstate, Upstate, and Stony Brook)
- University of Alabama, Birmingham
- University of California, Davis
- UCLA
- University of Cincinnati
- University of Colorado, Denver
- University of Kansas
- University of Miami
- University of Pittsburgh
- University of Rochester
- University of Utah
- University of Virginia
- University of Texas, Dallas
- Vanderbilt
- Washington University in St. Louis

# NeuroNEXT



CCC-Mass General Hospital  
DCC-University of Iowa

# Working with NIH intramural

- We cannot give extramural funds to intramural investigators BUT if an NIH intramural investigator decides to collaborate with an extramural investigator of his/her choice, the study can be funded through NeuroNEXT with work being done both internal and external to NIH.
- Clinical center may become an *ad hoc* site

# Working with consortia

- Complementary efforts with each group working within its own strengths
- NeuroNEXT does not conduct natural history studies
- Natural history studies may reveal possible areas of intervention that could be evaluated in a short-term limited Phase II trial—which NeuroNEXT could consider for implementation
- NeuroNEXT studies subject to NIH data –sharing policy.

# Existing Trial Groups & NeuroNEXT

- Expertise of existing trial groups is welcomed for Phase II studies in NeuroNEXT
- A PPI or Co-PI from an existing trial group can submit a concept proposal, but would need to use the NeuroNEXT clinical study sites if the study was funded
- If an existing trial group wants to use all NN sites in addition to several non-NN sites, a justification would be needed:
  - Need for specific patient population
  - Need for clinical expertise in a disease area

# Working with advocacy

- All NeuroNEXT Protocol Working Groups include a patient representative to provide input on the protocol as well as the informed consent from the very beginning.

# Applying to NeuroNEXT-U01

- Three different mechanisms for applying to NeuroNEXT:
  - U01 (PAR 13-343)
  - X01 (PAR 11-344)
  - U44 (PAR 11-345)
- The only FOA that has been revised is the one for the **U01** mechanism: PAR 13-343

# Who is eligible to apply?

- ANYONE may apply
- You do not have to be based at a NeuroNEXT site to apply.
- If your proposal is approved for grant funding, your site becomes an *ad hoc* NeuroNEXT site for the duration of the study/trial.

# Revised U01 FOA: **Scope**

- The SCOPE section has been expanded.
  - Studies to determine clinical outcome measures for a given condition are within scope
  - Studies to evaluate potential biomarkers-even if no investigational agent is involved-are within scope
  - Comparative effectiveness trials that aim to select the best product for a future study are within scope

# Revised U01 FOA: Other KEY changes

- Implementation (section I)
  - New information on device studies in NeuroNEXT
  - #6 on rationale (note the last sentence of the paragraph)
- Other attachments(section IV)
  - A file with regulatory documents must be included
  - Expected enrollment timelines must be discussed
- Criteria-review discussions(section V)
  - Part 1: focus on scientific rationale/premise
  - Part 2: clinical aspects of the study and the listed review criteria

# Current NeuroNEXT studies

- NN 101: Spinal Muscular Atrophy (SMA) Biomarkers in the Immediate Postnatal Period of Development (Stephen Kolb MD PhD, Ohio State University)
- NN102: Secondary and Primary progressive Ibudilast NeuroNEXT Trial in Multiple Sclerosis – SPRINT-MS (Robert Fox MD, Cleveland Clinic)
- NN103: A Phase 2 trial of Rituximab in Myasthenia Gravis (Richard Nowak MD, Yale)

# Epilepsy proposals received to date

- Year 1 (9/11-8/12): Eight
- Year 2 (9/12-8/13): Three
- Year 3 (9/13-now): None

[ Website and contact information

[www.neuronext.org](http://www.neuronext.org)

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