

## Update on Success and Five-Year Renewal Application

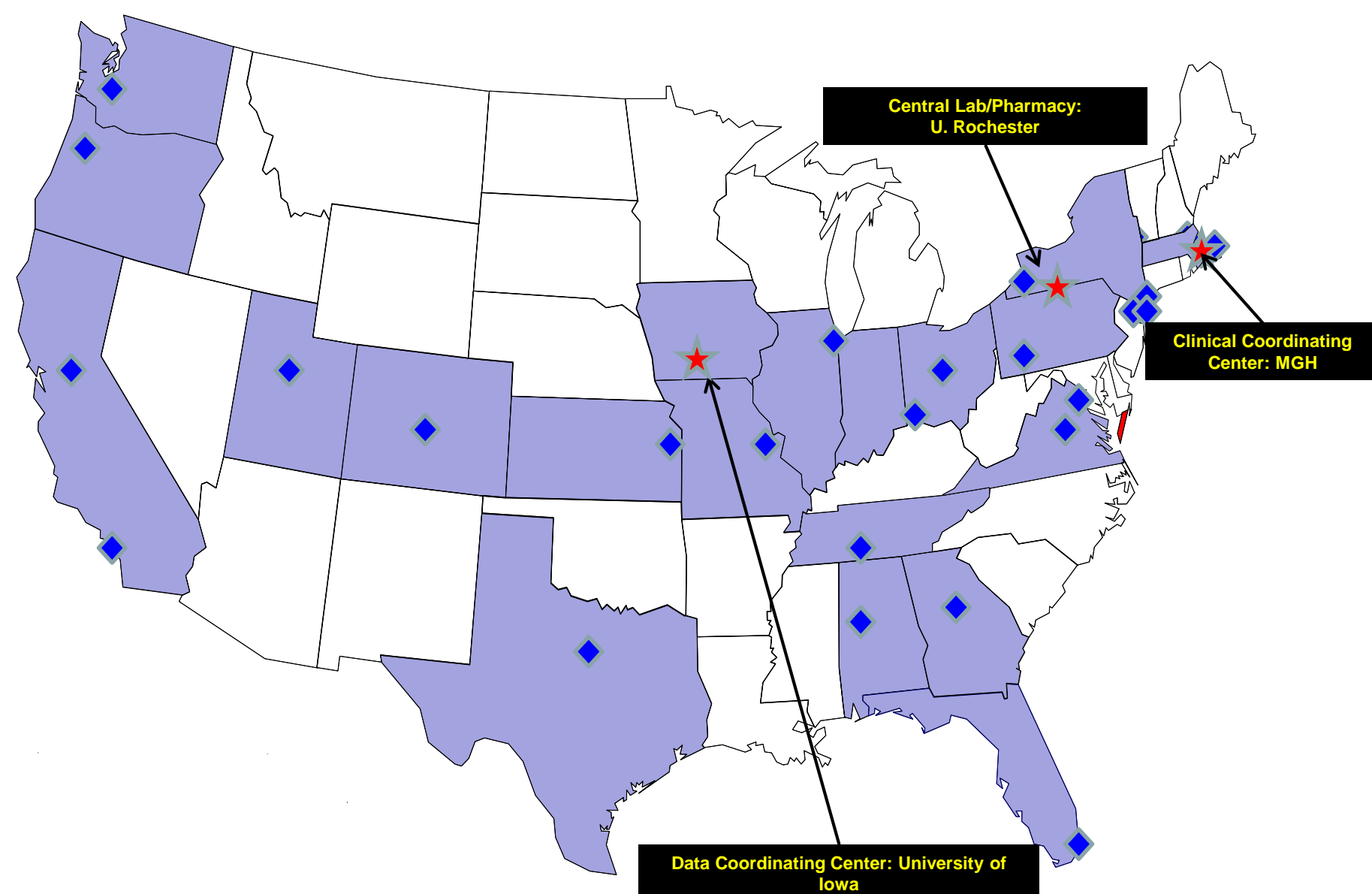
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### Background

- ❖ NeuroNEXT (NN), an NIH-funded network was designed to:
  - Increase efficiency of clinical trials
  - Expand NINDS' capability to test promising new therapies
  - Respond quickly as new opportunities arise to test promising new treatments for people with neurological disorders
- ❖ The Network accepts proposals for phase 2 clinical studies from academia, industry and foundations – [www.neuronext.org](http://www.neuronext.org)
- ❖ NINDS established 3 funding mechanisms: for academic investigators (U01), small businesses (U44) and industry collaborators (X01)
- ❖ Three Key Initiatives of the NeuroNEXT Network:
  - Establishment of a Central IRB - All participating institutions required to establish a Reliance Agreement with the Partners IRB.
  - Establishment of Master Clinical Trial Agreements (MCTA) – All participating institutions required to establish a MCTA with the Clinical Coordinating Center. Separate site agreements are not required for studies conducted by NN
  - Availability of experienced clinical trial clinicians and statisticians to help with study design activities

### Clinical Study Sites



### Network Success (as of 2/16/18)



Rich Pipeline

- 9 studies funded to date
- 4 completed; 4 actively enrolling; 1 in start-up



Operational Efficiency

- Decreased study start up time
- Meeting study recruitment and retention targets
- Innovative study design
- Exceptional data quality

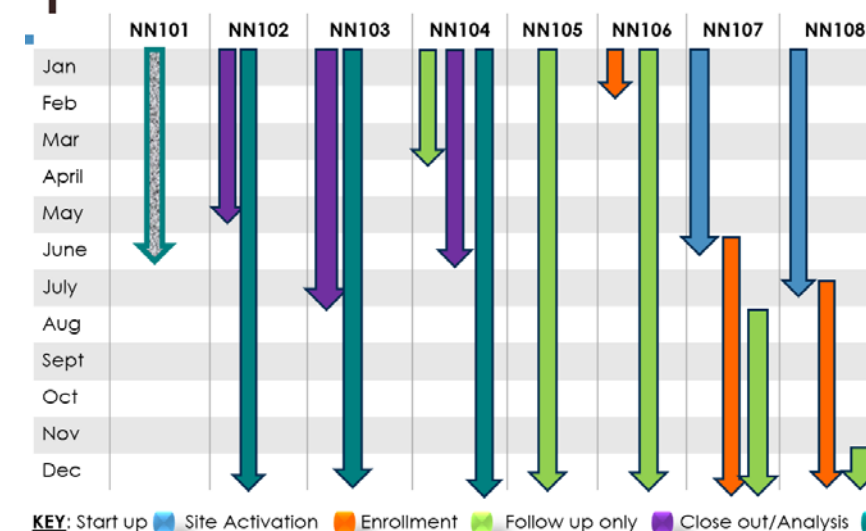


Network Partnerships

- Expanded number of trained investigators
- Built cohesive network
- Partnerships with academics, foundations and industry

- ❖ **Spinal Muscular Atrophy Biomarker Study** (Kolb – Ohio State) U01
- ❖ **Multiple Sclerosis Intervention Trial** (Fox – Cleveland Clinic) U01
- ❖ **Myasthenia Gravis Intervention Trial** (Nowak - Yale) U01
- ❖ **Acute Stroke Intervention Trial** (Lyden – Cedars Sinai / Pryor – ZZ Biotech) X01
- ❖ **Huntington's Disease Intervention Trial** (Hersch MGH / Brownstein – Azevan) U44
- ❖ **Cytochrome C as Biomarker in Glioblastoma Multiforme** (Griguer – UAB at Birmingham) U01
- ❖ **Fragile X Syndrome Intervention Trial** (Berry-Kravis – Rush University) U01
- ❖ **Topiramate for Cryptogenic Peripheral Neuropathy** (Smith—University of Utah) U01
- ❖ **ManNac for GNE Myopathy** (Carrillo– NIAMS) U01

#### 2017 Network Activities



### Key Achievements

- ❖ Three trials closed out simultaneously in late 2017.
- ❖ Average 3.6 months from funding to database release.
- ❖ All trials completed enrollment on time, with enrollment rates far exceeding condition-specific standards.
- ❖ 96% data accuracy (no changes required to CRFs).
- ❖ 80% of data entered within 7 days of collection; 94% in 30 days.
- ❖ 80% of subjects with no major protocol deviations.
- ❖ 85% retention rate.
- ❖ Success of cIRB model
  - ❖ Full protocol approval: average 64±25 days
  - ❖ Child site approval: average 17±9 days
  - ❖ Continuing review approval: average 16 days
- ❖ Electronic trial master file containing 21,000 documents for 1300 site personnel.
- ❖ Streamlined SAE review process, incorporating central DSMB
- ❖ Expanding the number of trained clinical investigators through in-person and web-based training.

### CONCLUSIONS

- ❖ NeuroNEXT has met all of its initial goals within 7 years of funding – conducting 8 studies, in partnership with industry (stroke), academia (SMA, MS, MG, GBM, FXS, CSPN) and small businesses (HD)
- ❖ Network sites have demonstrated their capacity to effectively enroll subjects in trials for a variety of neurologic diseases
- ❖ The NeuroNEXT Network is primed for future collaborations with industry, academia and small business partners. For more information about how to apply to NeuroNEXT, please visit our website, [www.neuronext.org](http://www.neuronext.org)

### ACKNOWLEDGEMENT

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